ORPHAZYME

Orphazyme A/S
CVR No.: 32266355
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ANNUAL REPORT 2020

# Advancing treatment in neurodegenerative rare diseases

**Lleyton Coombes**
Lleyton is 14 years old and suffers from Niemann-Pick disease Type C (NPC). Here he is pictured on set of award-winning Niemann-Pick UK (NPUK) short film **"Go Make Memories"** (2019). Lleyton was the only live-action actor featured in the production, with his story directly inspiring the film along with many other members of the NPUK Community.

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*Picture kindly supplied to Orphazyme A/S by the Kirk Family

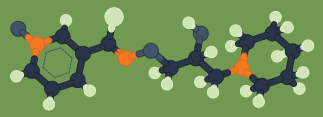
# AT A GLANCE

## Pioneering the Heat-Shock Protein response for neurodegenerative rare diseases

### APPROACHING COMMERCIALIZATION: FIRST POTENTIAL APPROVAL IN 2021

Completed important key regulatory filings in US and EU for arimoclomol in NPC during 2020; eagerly awaiting decisions

### ARIMOCLOMOL



- First-in-class Heat-Shock Protein (HSP) amplifier
- Breakthrough Designation in NPC
- Orphan Drug & Fast Track Designations in NPC, ALS, IBM

### PIPELINE-IN-A-PRODUCT OPPORTUNITY

Arimoclomol's current programs could target

**~100K** patients in NPC, ALS, IBM, & Gaucher

### 2 PIVOTAL RESULTS DUE H1 2021

Amyotrophic Lateral Sclerosis (ALS)

Inclusion Body Myositis (IBM)

**DKK 727M**

**(USD 119M)**

cash at year-end 2020

### EXPANDING OUR GLOBAL FOOTPRINT AHEAD OF LAUNCH

Global HQ: **Copenhagen, Denmark**

US: **Chicago, USA**

International: **CH, DE, FR**

Dual listing on Nasdaq:
**ORPHA.CO | ORPH.US**

**141**
**Total employees**

**33**
**FTEs in pre-launch & commercial**

# ACHIEVEMENTS

## 2020

### JANUARY
Positive arimoclomol open-label Phase 2/3 extension data in NPC

### JANUARY
Launched US EAP[1] for NPC

### FEBRUARY
Completed financing of approx. DKK 745 million (USD 110 million)

### MAY
U.S. Fast Track Designation for arimoclomol in ALS

### JUNE
Arimoclomol Phase 2 Gaucher disease data demonstrating marked improvements in key clinical markers

### SEPTEMBER
U.S. FDA acceptance and Priority Review of NDA[2] for arimoclomol for NPC

### OCTOBER
Completed global offering and U.S. IPO raising approx. DKK 534 million (USD 84 million)

### NOVEMBER
Submitted European Marketing Authorisation Application for arimoclomol for treatment of NPC

### NOVEMBER
Established US main office in Chicago, Illinois, USA

## 2021

1. Early Access Program
2. New Drug Application

LETTER FROM THE CHAIRMAN

# BRINGING AN INNOVATIVE THERAPY TO PATIENTS IN URGENT NEED

**All of us at Orphazyme are inspired by the opportunity to profoundly impact the communities we serve. As we eagerly anticipate the first approval of our Heat-Shock Protein amplifier – arimoclomol – we are focused and ready to deliver this innovative treatment to the Niemann-Pick disease Type C (NPC) community and remain committed to expanding its potential in additional underserved diseases.**

**This is a truly momentous time at Orphazyme. The company is poised to bring its innovative Heat-Shock Protein (HSP) response therapy, arimoclomol, to the NPC community. Further, we are closer than ever before in understanding the potential of arimoclomol in advancing care for people with other rare neurodegenerative diseases. The hard work of so many contributors helped Orphazyme achieve critical milestones that will shape the company's future.**

- Orphazyme successfully completed the submission of two key regulatory applications to the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for arimoclomol in its first indication of NPC

- Orphazyme advanced its promising pipeline programs with trial progress and data presentations that shape the community's understanding of the Heat-Shock Protein response. The potential of arimoclomol in new areas was reinforced by promising Phase 2 data in Gaucher disease and with the receipt of the FDA's Fast Track Designation for the treatment of Amyotrophic Lateral Sclerosis (ALS)
- All of this has been reinforced by a successful financing round and an initial public offering (IPO) in the U.S. This dual listing with Nasdaq Copenhagen and U.S. strengthens the company's financial position and supports its expansion plans for the future

In December 2020, Orphazyme parted ways with former Chief Executive Officer, Kim Stratton. The Board appreciates Ms. Stratton's important contributions that have helped put the company on track to becoming a global commercial-stage company. Orphazyme is well-positioned for growth; in addition to its assets in development, the company has an executive team that brings a wealth of rare disease experience across geographies and expertise, spanning clinical development to commercialization. Orphazyme's employees are connected by a set of core values focused on care, courage, perseverance, and integrity. The company's close ties to the research, medical, and patient communities underscore the sense of urgency and commitment to progress arimoclomol as an innovative therapy for underserved diseases. Despite the challenges faced by all of us as a result of the ongoing COVID-19 pandemic, the Orphazyme team had a very productive 2020 and is closer than ever to delivering on its ambition to bring arimoclomol to patients.

Now, with so much incredible momentum and a strong financial position, Orphazyme looks forward with purpose and optimism to 2021 and beyond.

Orphazyme's first priority for this year is to deliver on its mission to serve the patient communities that have been eagerly awaiting new solutions. Should the company receive regulatory approvals, it will have the opportunity to bring arimoclomol to the NPC communities in the U.S. and Europe. The company has established an Early Access Program in the U.S., France, and Germany to provide access for eligible patients until arimoclomol is approved. The company is exploring options to offer similar programs in additional countries in Europe. In line with the company's growth plans, it will continue to invest in its manufacturing and operational footprint.

Beyond NPC, Orphazyme is expecting data readouts from two pivotal trials of arimoclomol in ALS and Inclusion Body Myositis (IBM) during the first half of 2021. Further, Orphazyme continues to invest in its research on the Heat-Shock Protein response and lysosome biology, where the team is actively working to develop the next generation HSP amplifiers and lysosome biology-targeted compounds. We are confident that these efforts will support the company's ambition to bring innovative new therapies to patients.

On March 1, 2021, we announced Christophe Bourdon will join Orphazyme as Chief Executive Officer, starting April 1, 2021. Christophe has a strong track record in launching and commercializing rare and non-rare disease products in Europe and the United States, with recent senior positions at Amgen and Alexion. We are delighted to have him lead the company into our next growth phase.

I am privileged to have been involved in this journey during my tenure as a member of the Board of Directors and as Chairman, since 2014, and to witness this successful transition. On behalf of the Board of Directors, I would like to thank our shareholders, employees, partners, and the many members of the community, including patients and families, physicians and researchers, and patient organizations, who have provided critical perspectives and guidance that has shaped our journey. Without your continued trust and support, none of this would be possible.

Best regards

Georges Gemayel
Chairman of the Board of Directors

# KEY FIGURES

| (TDKK) | 2020 | 2019 | 2018 | 2017 | 2016 |
|---|---|---|---|---|---|
| **Statement of profit and loss and other comprehensive income** | | | | | |
| Research and development expenses | (361,284) | (285,413) | (196,525) | (99,048) | (55,817) |
| General and administrative expenses | (247,250) | (50,541) | (35,127) | (31,994) | (7,703) |
| **Operating loss** | **(608,534)** | **(335,954)** | **(231,652)** | **(131,042)** | **(63,520)** |
| Net financial items | (26,627) | (7,043) | (3,448) | (662) | 85 |
| **Loss before tax** | **(635,161)** | **(342,997)** | **(235,100)** | **(131,704)** | **(63,435)** |
| Income tax benefit | 1,915 | 5,500 | 5,500 | 5,500 | 5,500 |
| **Net loss for the period** | **(633,246)** | **(337,497)** | **(229,600)** | **(126,204)** | **(57,935)** |
| **Total comprehensive loss** | **(632,641)** | **(337,430)** | **(229,558)** | **(126,204** | **(57,935)** |
| Loss per share, basic (DKK) | (22.32) | (16.85)* | (11.47)* | (10.43) | (5.89) |
| **Statement of financial position** | | | | | |
| Intangible assets | 12,454 | 10,539 | 10,744 | 9,853 | - |
| Right-of-use assets | 14,859 | 13,903 | - | - | - |
| Property, plant, and equipment | 4,687 | 3,685 | 1,940 | 1,851 | 1,225 |
| Total non-current assets | **38,829** | **32,529** | **17,965** | **14,864** | 4,047 |
| Cash | 726,929 | 123,588 | 394,706 | 631,735 | 14,349 |
| Other current assets | 56,735 | 24,637 | 28,678 | 16,218 | 13,545 |
| Total assets | **822,493** | **180,754** | **441,349** | **662,817** | **31,941** |
| Share capital | 34,698 | 19,984 | 19,939 | 19,928 | 3,361 |
| Total equity | 620,525 | 52,969 | 388,249 | 615,702 | 17,509 |
| Non-current borrowings | 23,830 | 51,606 | - | - | - |
| Non-current lease liabilities | 9,877 | 9,813 | - | - | - |
| Total current liabilities | 166,627 | 65,988 | 52,995 | 47,115 | 14,432 |

* Adjusted retrospectively, see Note 4.3 in consolidated financial statements

| (TDKK) | 2020 | 2019 | 2018 | 2017 | 2016 |
|---|---|---|---|---|---|
| **Cash flow statement** | | | | | |
| Cash flow from operating activities | (539,076) | (326,818) | (234,764) | (95,426) | (54,724) |
| Cash flow from investing activities | (5,101) | (3,285) | (2,346) | (1,491) | (238) |
| Cash flow from financing activities | 1,159,422 | 58,939 | - | 714,303 | 1,300 |
| **Other** | | | | | |
| Share price (DKK)[1] | 67.10 | 72.40 | 43.35 | 76.00 | - |
| Total outstanding shares | 34,697,703 | 19,984,799 | 19,939,564 | 19,928,184 | 3,360,541 |
| Market capitalization (MDKK)[2] | 2,328.2 | 1,446.9 | 864.4 | 1,514.5 | - |
| Equity ratio[3] | 75.4% | 29.3% | 88.0% | 92.9% | 54.8% |
| Equity per share (DKK)[4] | 17.88 | 2.65 | 19.47 | 30.90 | 5.21 |
| Average number of employees | 117 | 74 | 46 | 26 | 17 |
| Number of employees at the end of the year | 141 | 86 | 57 | 34 | 21 |

The comparatives figures for 2018-2015 have not been restated following the adoption of IFRS 16 *Leases* on January 1, 2019 by use of the modified retrospective approach.

(1) There is no official share price for the reporting periods prior to 2017 since the Company only went public in 2017
(2) Market cap is calculated as the share price multiplied with the total outstanding shares as of the balance sheet date
(3) Equity ratio is calculated as the equity divided by the total assets as of the balance sheet date
(4) Equity per share is calculated as the total equity divided by the total outstanding shares as of the balance sheet date

# FINANCIAL REVIEW

## INCOME STATEMENT

The net loss for the financial year ended December 31, 2020 was DKK 633.2 million compared to DKK 337.5 million for the same period in 2019. The increased net loss was primarily driven by a continued investment in research and development activities, escalation of our commercial launch preparations and strengthening of our global team.

## RESEARCH AND DEVELOPMENT EXPENSES

Research and development expenses for the year ended December 31, 2020 were DKK 361.3 million compared to DKK 285.4 million for the year ended December 31, 2019. The increase of DKK 75.9 million was mainly attributable to costs related to three clinical pharmacology registrational trials that ramped up and took place mainly during 2020 in addition to increased clinical safety reporting activity in the ongoing clinical trials. In addition, our employee costs increased as a result of 12 more full-time research and development employees hired during 2020.

## GENERAL AND ADMINISTRATIVE EXPENSES

General and administrative expenses for the year ended December 31, 2020 were DKK 247.3 million compared to DKK 50.5 million for the year ended December 31, 2019. The increase of DKK 196.7 million was primarily due to the build-up of our commercial organization as well as expenses related to increased costs for being a listed company in the U.S.

Pre-launch expenses represented DKK 112.2 million of the increase, which was mainly due to the escalation of commercial launch preparation activities, including the strengthening of our U.S.-based and Switzerland-based commercial team by hiring 29 full-time employees. We also significantly increased our medical affairs activities, particularly for NPC, as we further engaged with the scientific community through our communication and education programs.

Administrative expenses represented the remaining DKK 84.5 million increase, which was mainly due to share-based compensation expenses attributed to long-term incentive programs; audit, legal, investor relations, other external assistance engaged to support us in the requirements of being a listed entity in the U.S; and the hiring of 20 additional administrative, finance, legal and IT full-time employees to support our growing organization.

## NET FINANCIAL ITEMS

Net financial expenses for the year ended December 31, 2020 were DKK 26.6 million compared to DKK 7.0 million for the year ended December 31, 2019. The increase of DKK19.6 million was mainly related to an increase in net foreign currency exchange losses of DKK 13.0 million primarily due to a decrease in the US dollar versus DKK, as well as increased interest expenses related to the Loan Agreement with Kreos of DKK 6.6 million.

## INCOME TAX BENEFIT

Income tax benefit for the year ended December 31, 2020 was DKK 1.9 million compared to DKK 5.5 million for the year ended December 31, 2019. Income tax benefit for the two periods include a tax credit for research and development costs at the applicable tax rate under the Danish Corporate Income Tax Act. The amount of the tax benefit in the year 2020 has been reduced by an income tax expense in our subsidiaries in the U.S. and Switzerland. Our corporate income tax rate in Denmark was 22%. However, for the year ended December 31, 2020 and 2019, we did not recognize any deferred tax assets in Denmark considering uncertainties surrounding their potential utilization.

## STATEMENT OF FINANCIAL POSITION

**Cash:** As of December 31, 2020, Orphazyme had cash of DKK 726.9 million compared to DKK 123.6 million as of December 31, 2019. The increase in cash was primarily due to net proceeds from our directed issue and private placement in February 2020 and our global offering and U.S. IPO in September 2020.

**Equity:** As of December 31, 2020, total equity amounted to DKK 620.5 million compared to DKK 53.0 million as of December 31, 2019. The increase mainly reflects the increase in cash.

## CASH FLOWS

**Cash flow from operating activities:**
Net cash used in operating activities for the period ended December 31, 2020 was DKK 539.1 million compared to DKK 326.8 million in the year ended December 31, 2019. The increased use of cash was primarily attributable to the progression of clinical development activities, in particular the clinical pharmacology registration trials, as well as commercial launch preparation activities.

**Cash flow from investing activities:**
Net cash used in investing activities for the period ended December 31, 2020 was DKK 5.1 million compared to DKK 3.3 million in the year ended December 31, 2019. The increase was mainly due to the investment in our new ERP system.

**Cash flow from financing activities:**
Net cash provided by financing activities for the period ended December 31, 2020 was DKK 1,159.4 million compared to DKK 58.9 for the year ended December 31, 2019. The increase of DKK 1,100.5 million reflects the net proceeds of DKK 694.2 million from our directed issue and private placement in February 2020 and DKK 477.9 million from our global offering and U.S. listing in October 2020.

# 2021 OUTLOOK

We expect 2021 to be a transformational year for Orphazyme with the anticipated approval and launch of arimoclomol in NPC and two key readouts from our late-stage clinical trials in IBM and ALS. The outcome and timing of these events are key factors in driving our guidance for 2021, along with other assumptions and risks which are outlined below.

Our 2021 outlook for operating loss, operating expenses, and cash position includes the following key assumptions:

- The approval of arimoclomol for NPC in the U.S. by the PDUFA action date of June 17, 2021;
- The grant of a Priority Review Voucher (PRV) upon such approval and our ability to sell the PRV voucher at generally-accepted market rates;
- Initial revenues from arimoclomol in the U.S. and named patient sales in certain countries;
- Approval of arimoclomol for NPC in Europe in Q4 2021;
- Continued investments in our commercial infrastructure in the U.S. and Europe to support product launches;
- R&D expenses to support the advancement and completion of arimoclomol clinical trials in IBM and ALS, including data readout and preparations for filing, if successful


## OPERATING LOSS

**100 – 150 DKK M**

16 – 24 USD M*

### 2021 guidance

Launch activities will be considerably influenced by the roll-out of our commercial strategy and potential market entries. We are well-prepared to launch arimoclomol for NPC within a narrow window of arimoclomol being approved in the U.S. and our forecast assumes a gradual increase in sales through the second half of 2021, reaching DKK 60 - 120 million (~$10 - 20 million) in revenues by year-end.

Our outlook assumes that a PRV is granted and that we are able to sell the voucher. The value of the PRVs sold during 2020 was between USD 95-110 million.

Our ALS and IBM trials for arimoclomol are expected to read out in H1 2021. Depending on the results and discussions

## OPERATING EXPENSES

**800 – 850 DKK M**

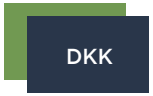
130 – 138 USD M*

### 2021 guidance

with regulators, we plan to accelerate our filing activities.

Our R&D expenses include costs for ongoing clinical trials including extensions and investment in our new molecular entity program of next generation HSP amplifiers, which are currently in lead optimization.

Management monitors the Company's funding risks and liquidity needs, with a view to continue as a going concern. Subsequently, funding options are evaluated on a continuous basis, including public or private debt and equity financing, in order to ensure sufficient funding to continue ongoing clinical trials and commercial launch activities.




## CASH POSITION AT YEAR-END

**>350 DKK M**

> 57 USD M*

### 2021 guidance

Should the Company find itself unable to attain funding, the Company may down-size or delay planned activities to allow funds to last until December 31, 2021. Management therefore considers it appropriate to prepare these financial statements on a going concern basis.

In addition to the key factors mentioned above, our outlook is subject to various risks and uncertainties, including but not limited to the timing of regulatory decisions, the success of our commercial efforts and our development activities. Further details on risks associated with our business are outlined in the risk management section of this report on pages 30-31 and in our Annual Report on Form 20-F filed with the SEC.

*USD figures are for reference only; FX rate 1 DKK/0.16 USD

# PRIORITIES 2021

**2021 WILL BE A TRANSFORMATIONAL YEAR FOR ORPHAZYME, WITH POTENTIAL APPROVAL OF ARIMOCLOMOL IN NPC AND PIVOTAL DATA READOUTS IN ALS AND IBM.**

Our top priority in the immediate term is securing approval of arimoclomol in NPC in the U.S. and Europe, so we can serve the NPC patient community that has been eagerly awaiting a new treatment option for this devastating disease.

Our commercial team is fully in place in the U.S. and we have almost completed our preparations for launch of arimoclomol in Europe, where we expect to gain approval in Q4 2021.

We look forward to announcing the outcome of our pivotal trials in ALS and IBM. Positive results from these trials would reinforce our long-term strategy to expand arimoclomol beyond NPC to additional neurodegenerative orphan diseases with significant unmet treatment needs.

**ALS:**
**Phase 3 top-line data**

**H1 2021**

**IBM:**
**Phase 2/3 top-line data**

**H1 2021**

**NPC:**
**Potential U.S. approval and launch**

**Target PDUFA date: June 17, 2021**

**NPC:**
**Potential MAA approval in EU**

**Q4 2021**

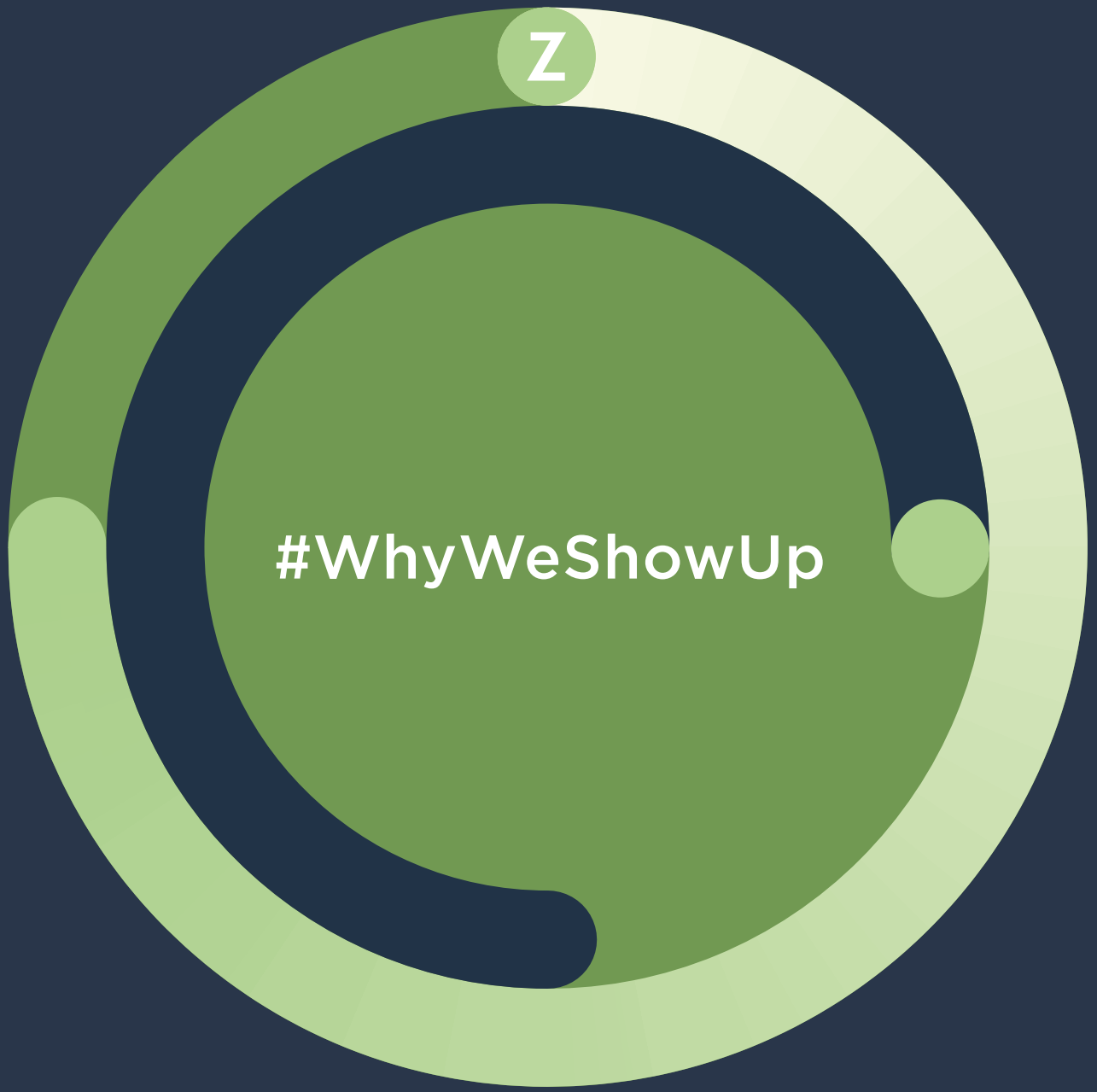
**We are fiercely committed to providing new therapeutic solutions for patients living with neurodegenerative diseases.**

## WHY DO WE SHOW UP?

It is an important question. We are all part of the Orphazyme team because of a shared energy and a shared vision. We are on the verge of making a real difference for people living with rare, orphan diseases.

These are people who have waited far too long for an approved therapy. We get the chance to change that by supporting these patients, deepening our connection to them, and making sure they do not face their journey alone.

This is #WhyWeShowUp. Even if we each show up for slightly different reasons, the important thing is that we do.



SPOTLIGHT

# THE KIRK FAMILY JOURNEY IN NPC

**Graham Kirk was an active 25-year-old, living a vibrant life in Ireland; playing sports, starting his dream job, and dating the love of his life, Ciara. However, in September 2010, his life suddenly changed forever. Graham was diagnosed with Nieman-Pick disease Type C (NPC), a rare, neurodegenerative disease with no cure. NPC is a hereditary lysosomal disease that can affect the brain, nerves, spleen, and other organs, and can be fatal.**

Graham Kirk and his family

Picture kindly supplied by the Kirk Family to Orphazyme A/S.

> "NPC is often difficult to diagnose because symptoms and age of onset vary greatly by individual

For years, Graham's sister Paula had suffered from a mysterious condition that affected nearly every aspect of her wellbeing – both physical and mental – and her health progressively worsened over time. NPC is often difficult to diagnose because symptoms and age of onset vary greatly by individual, even for people in the same family. When Paula finally received a diagnosis of NPC, it was suggested by her doctor that Graham also be tested, due to the genetic nature of the condition.

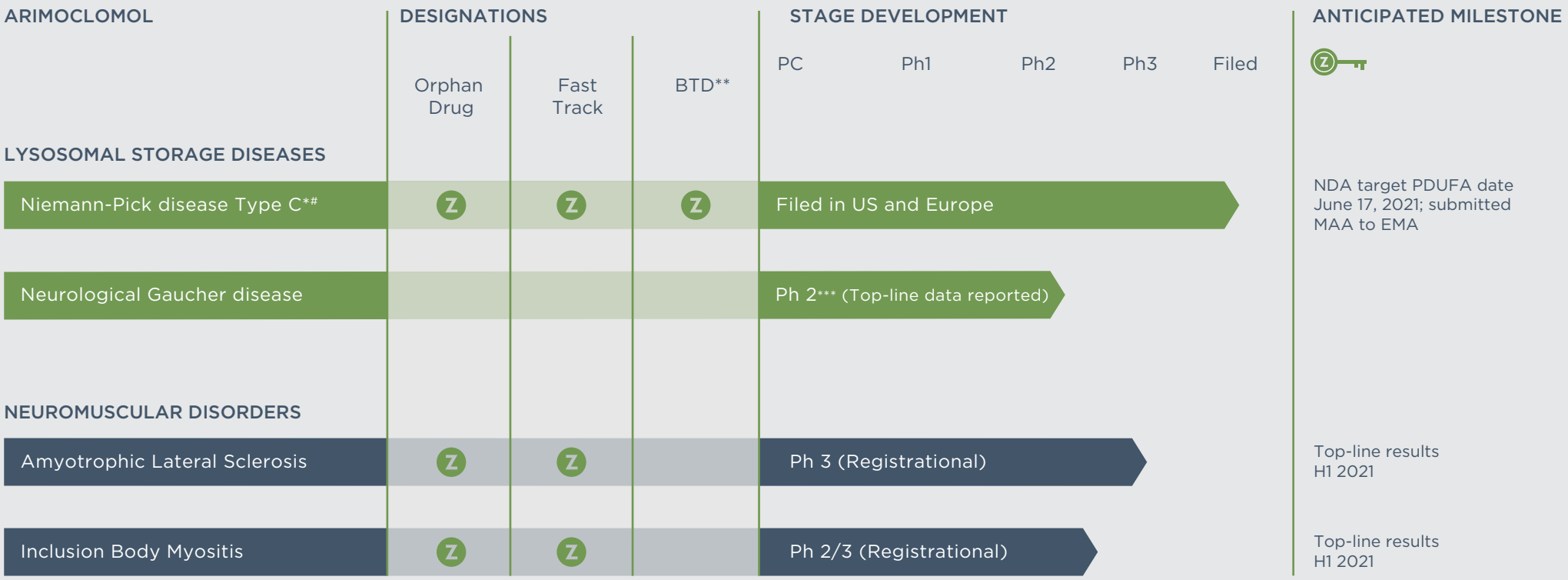
Graham was shocked to learn that he, too, had NPC. At that time, an inability to move his eyes up and down were his only noticeable symptoms. But he well understood the devastating effects of the disease as it progresses. Paula's condition profoundly impacted their family's life, which is a shared reality for many parents and family caregivers whose loved ones have NPC.

In the face of a daunting diagnosis, Graham and Ciara remained strong. Their first action was to seek support and information from Niemann-Pick UK, an organization working diligently to raise awareness of NPC and provide information, connection, and hope to those living with the disease. As they learned more about what the future might hold, the couple agreed that NPC would not define their lives; rather, together they would make their decisions around it; embracing new experiences and building lasting memories.

Today, Graham has reduced strength and cramps in his hands, muscle spasms, and was recently prescribed hearing aids, but considers himself lucky that his symptoms are manageable. Sadly, Paula passed away in 2012, and her family continues to advocate for greater awareness and progress against NPC in her memory. And with new innovation emerging, patients like Graham around the world have hope for a brighter future.

Today, Graham and Ciara are married and quite busy raising three beautiful young children. Graham is also a golfer, a devoted friend, an uncle, and a godfather, who happens to live with NPC. The Kirks are living every day to the fullest and sharing their story to inspire others to do the same.

# PRODUCT PIPELINE

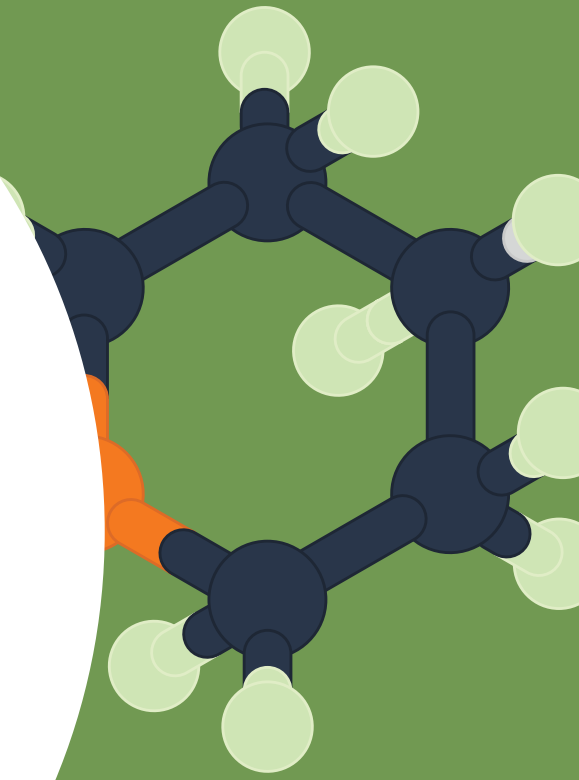



* Early-access programs in the US, France and Germany
** Breakthrough Therapy Designation (BTD)
*** Type 1 and Type 3 Gaucher disease
# Rare Pediatric Disease Designation

# Arimoclomol selectively amplifies Heat-Shock Proteins (HSPs), the cell's natural defense system

HSPs help to protect cells under stress from the accumulation of misfolded proteins, aggregated proteins and dysfunctional lysosomes which could otherwise lead to toxicity and disease.

Learn more about our science at www.orphazyme.com



ARIMOCLOMOL

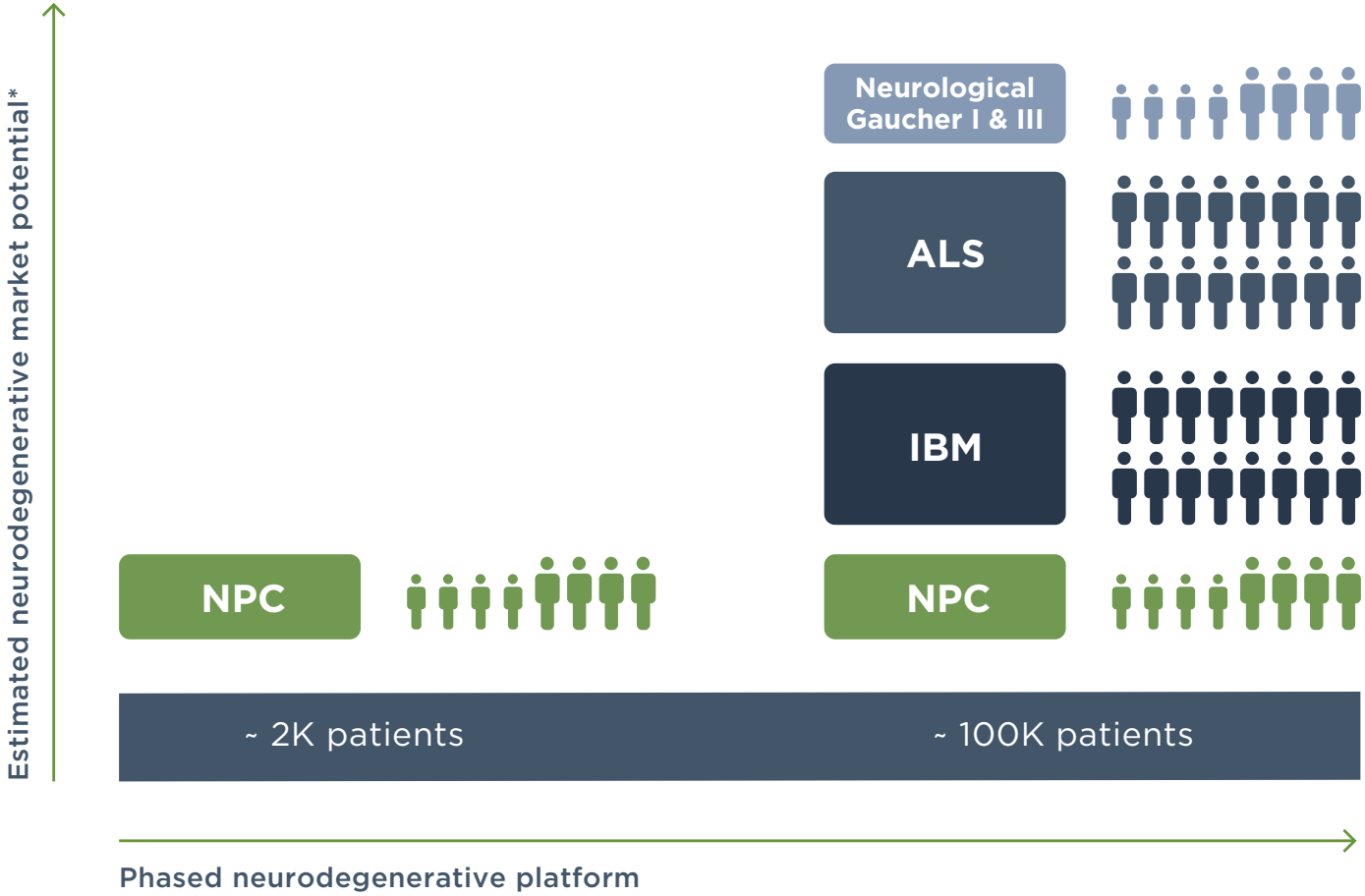
# PIPELINE-IN-A-PRODUCT OPPORTUNITY

## ARIMOCLOMOL IS A FIRST-IN-CLASS HEAT-SHOCK AMPLIFIER WITH SIGNIFICANT POTENTIAL IN A RANGE OF DISORDERS.

We are pioneering the use of a natural cellular defense system, the heat shock response, or Heat-Shock Protein (HSP) response, for the treatment of neurodegenerative orphan diseases.

We believe arimoclomol is the first clinical product to harness this mechanism for lysosomal and neuromuscular disorders, and that it could have broad potential where misfolding and/or aggregation are hallmarks of disease.

Arimoclomol is currently in clinical development in four indications, with readouts or potential approvals expected in three of these during 2021. If successful, arimoclomol could help address significant unmet medical needs across a broad range of patients in the medium term.




* Estimated patients represent U.S. and Europe combined and figures are rounded.

PRODUCT PIPELINE

# NIEMANN-PICK DISEASE TYPE C

## OVERVIEW

Niemann-Pick disease Type C (NPC) is a rare, fatal neurodegenerative disease caused by genetic mutations that most often lead to misfolded variants of the NPC proteins.

Misfolded NPC protein does not function properly and is subject to rapid degradation. As a consequence, lipids, that would normally be cleared, build up in the lysosomes of cells throughout the body.

Accumulation of lipids in the tissues and organs, including the brain, leads to loss of cell function and organ damage. Neurologic involvement is common and results in progressive motor and cognitive impairment.

## PREVALANCE

NPC often appears in childhood but can appear at any age. The incidence of the disease is estimated to be 1 in 100,000 live births and it is estimated that there are ~1,800 patients across U.S. and Europe.

In more aggressive forms, NPC is frequently fatal by the time patients reach their twenties.

**~1,800**

**patients across U.S. and Europe***

*Aptis partners Market Research for Orphazyme.


## TREATMENT OPTIONS

The majority of current treatment options are palliative and are only directed towards the specific symptoms apparent in each individual (e.g. prescription of anti-seizure medications to prevent seizures).

Only one drug, Zavesca® (miglustat), is currently marketed for NPC and only in certain jurisdictions outside the U.S., but there is still a very large unmet need for new therapy in NPC.

## MUTATIONS IN NPC PROTEIN

NPC is a lysosomal storage disorder caused by mutations on one of two genes, NPC1 or NPC2. Approximately 95% of individuals with the disease have mutations in NPC1.

**Symptoms of NPC include:**

- Difficulties in swallowing
- Loss of speech and cognition
- Motor coordination and ambulation issues
- Impairment of intellectual ability
- Psychiatric disturbances
- Progressive memory loss

**95%**

individuals have mutations in NPC1

## REGULATORY STATUS

| Lysosomal Storage Disease | Phase | Upcoming milestones |
|---|---|---|
| Niemann-Pick disease Type C | Filed | Anticipated approval during 2021 |

Arimoclomol has completed a Phase 2/3 trial in patients with NPC and is currently under review with both the U.S. FDA and the European Medicines Agency.

## EARLY ACCESS PROGRAM

We commenced our US Early Access Program (EAP) in January 2020 and have received approvals to commence EAPs in Germany and in France. We are currently evaluating how to offer early access in additional countries over time.

PRODUCT PIPELINE

# AMYOTROPHIC LATERAL SCLEROSIS

## OVERVIEW

Amyotrophic Lateral Sclerosis (ALS), also called Lou Gehrig's disease, is a rare neuromuscular disease which is rapidly progressive and usually fatal within three to five years. The disease attacks the neurons responsible for controlling voluntary muscles, resulting in weakness in limbs and an effect on breathing, speaking, and swallowing, leading ultimately to progressive disability and death.

The cause of neuron damage is unknown, but several theories exist, including glutamate toxicity, protein misfolding, and oxidative stress.

## PREVALANCE

Incidence of ALS in the U.S. is estimated to be two per 100,000 within the general population and prevalence is estimated to be between five and seven cases within a population of 100,000 (ALS association), equating to approximately 20,000 patients in the U.S. and 30,000 patients in Europe.

**~50,000**

**ALS patients in the U.S. and Europe***

**5,000**

**Diagnosed with ALS each year in the U.S.**

*Estimated prevalence of between five and seven cases per 100,000; ALS association.


## TREATMENT OPTIONS

There are currently very limited treatment options available to ALS patients, namely Rilutek® (riluzole), which was approved more than 20 years ago, and Radicava® (edaravone), which is only available in certain countries.

**A high unmet medical need remains for new therapies for ALS patients.**

## FAMILIAL AND SPORADIC ALS

**90%**

The majority of cases of ALS have no identified genetic component (sporadic ALS).

**10%**

Amongst familial ALS cases, 10% harbor mutations in a SOD1 enzyme.

Arimoclomol has so far been tested in two Phase 2 ALS trials:

- One **dose-ranging trial** in sporadic ALS
- One **trial in ALS** caused by SOD1 mutations

## TRIAL STATUS

| Neuromuscular disorders | Phase | Upcoming milestones |
|---|---|---|
| Amyotrophic Lateral Sclerosis | Ph 3 | Expected top-line results in H1 2021 |

Orphazyme initiated a Phase 3 trial in August 2018 to support the application for a marketing authorization in ALS. The trial is an 18-month, placebo-controlled trial including 245 patients. The primary endpoint is a combined assessment of function and survival (CAFS).

PRODUCT PIPELINE

# INCLUSION BODY MYOSITIS

## OVERVIEW

Inclusion Body Myositis (IBM, also referred to as sporadic IBM (sIBM)) is an acquired, rare, and slowly progressive muscle disorder, which typically affects men and has an average age of onset after age 50.

Many patients with IBM suffer from loss of fine motor skills such as writing, grooming, and the ability to eat unaided, and it is associated with significant morbidity, including a propensity to fall, difficulty swallowing, and severe disability. Up to three of every four cases of IBM occur in men.

## PREVALANCE

The prevalence of IBM has been estimated to be 4.6 per 100,000 people, equating to an estimated 40,000 individuals living with IBM in the U.S. and Europe combined.

**4.6 per 100,000**
Estimated prevalance*

**40,000**
Living with IBM in the U.S. and Europe

*Estimated prevalence of 4.6 per 100,000 people; Callan et al., Journal of Neuromuscular Diseases 4 (2017)


## TREATMENT OPTIONS

The standard treatment option for IBM consists only of supportive therapy (physical, speech, and occupational).

**There is currently no approved pharmaceutical treatment for IBM.**

## CAUSE OF IBM

In most cases, IBM progresses relentlessly over 10-15 years until the patient has lost mobility entirely and requires a wheelchair.

The cause of IBM is not fully known, but degenerative factors, i.e. the build-up of tangled and misfolded proteins (inclusion bodies), play a major role.

**10-15 years**
Until it is time for a wheelchair

**5 years**
May require walking stick as early as 5 years after symptom onset

## TRIAL STATUS

| Progressive muscle disorder | Phase | Upcoming milestones |
|---|---|---|
| Inclusion Body Myositis | Ph 2/3 | Expected top-line results in H1 2021 |

A multicenter, randomized, double blind, placebo-controlled Phase 2/3 trial in patients with IBM was initiated in August 2017. Intended to support the registration of arimoclomol for the treatment of IBM, this is a 20-month trial with IBMFRS (the Inclusion Body Myositis-Functional Rating Scale) as a primary endpoint.

PRODUCT PIPELINE

# GAUCHER DISEASE

## OVERVIEW

Gaucher disease is an inherited metabolic disorder caused by mutations in a protein called glucocerebrosidase, which leads to the accumulation of certain sugar-containing lipids.

The usual symptoms of Gaucher disease include an abnormally enlarged liver and/or spleen (hepatosplenomegaly), low levels of circulating red blood cells (anemia), blood cells promoting clotting (thrombocytopenia), and skeletal abnormalities.

## PREVALANCE

It is estimated that there are up to 15,000 Gaucher disease patients in the U.S. and Europe combined. Our focus is on addressing patients with neurological Gaucher disease.

**15,000**
**patients across U.S. and Europe***

*Estimated frequency of 1:40,000 - 60,000 live births; Siebert et al., Brain (2014)


## TREATMENT OPTIONS

The systemic symptoms of Gaucher disease are generally treated by existing enzyme replacement therapy, or ERT, and substrate reduction therapy, or SRT.

**There is currently no available treatment for neurological Gaucher disease.**

## TYPES OF GAUCHER DISEASE

**There are three main subtypes of Gaucher disease distinguished, in part, by the presence or absence of neurological symptoms such as muscle rigidity, loss of movement, seizures, cognitive impairment.**

**Type 1** is the most common form of Gaucher disease, with an estimated 30% of Type 1 patients going on to develop neurological symptoms later in life.

**Type 2** present with acute neurological symptoms

**Type 3** develop chronic neurological disease

**30%**
of Type 1 patients develop neurological symptoms

## TRIAL STATUS

| Lysosomal Storage Disease | Phase | Upcoming milestones |
|---|---|---|
| Gaucher disease | Ph 2 | Explore pivotal-stage |

In June 2020, results from a phase 2 study demonstrated marked improvements in key clinical markers, including liver and spleen size, over the initial six-month treatment period.

SPOTLIGHT

# LAUNCH PREPARATIONS

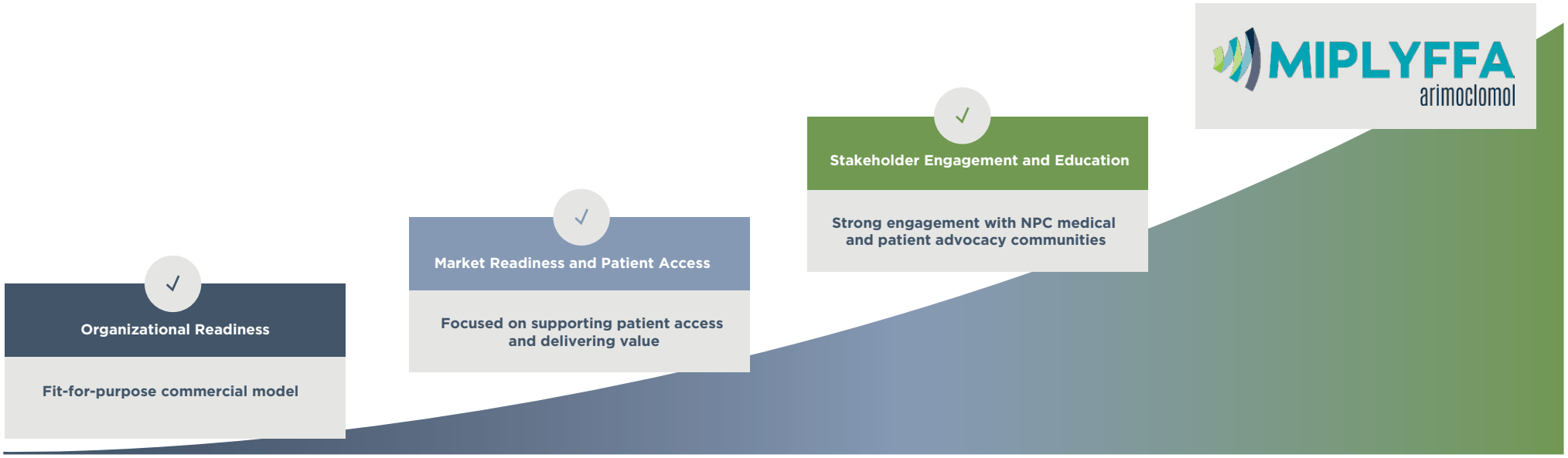
**OUR TOP PRIORITY IS TO ENSURE WE ARE READY TO SERVE NPC PATIENTS WITHIN A NARROW WINDOW OF ARIMOCLOMOL BEING APPROVED.**

Our team has significant expertise in the rare disease field and is working at pace globally on organizational readiness; market and patient access and stakeholder engagement and education to achieve this goal.

We made substantial progress in 2020 in preparing for potential approval of arimoclomol. In the U.S., we established a fit-for-purpose commercial model, headquartered in Chicago, with nearly thirty industry experts.

Our U.S. team has been working closely with regulators throughout the ongoing FDA review process and, among other launch preparations, has conducted extensive value and pricing research; engaged with payers; secured product supply and continued to bolster stakeholder engagement, disease awareness, and education efforts.

In Europe, we filed an MAA with EMA in November 2020. We continued to build our organization with the hiring of General Managers for France/Benelux, DACH, and Southern Europe and expanded our partnerships with rare disease experts in RoW. We also increased our patient access efforts and reimbursement dialogue in key European markets and continued our exchange with advisory boards, KOLs, and healthcare providers.

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MIPLYFFA
arimoclomol

We are proud to announce the global brand name for arimoclomol

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# PARTNERSHIPS

## UNIVERSITY OF KANSAS AND UNIVERSITY COLLEGE LONDON

**License Agreement**

In October 2017, Orphazyme entered into a license agreement with University of Kansas and UCL Business PLC (a wholly-owned subsidiary of University College London). The license agreement grants Orphazyme the global, royalty-bearing, exclusive license to develop and commercialize products under all data, know-how, inventions, and patent rights generated in the course of the on-going Phase 2/3 clinical trial of arimoclomol for the treatment of IBM.

Under the terms of the license agreement, Orphazyme shall pay an aggregate low single-digit royalty of net sales of products sold for the treatment of IBM for a period of 10 years from the first commercial sale in each country. The license agreement also provides that Orphazyme in consideration of the license shall issue bonus shares in favor of the University of Kansas and UCL Business PLC, for up to an aggregated value of USD 2.5 million in total depending on the size of the grants awarded to the universities under the trial (with a price per share calculated based on the average closing price of the shares on Nasdaq Copenhagen for the 30 days immediately prior to the date of issuance). The shares shall be issued or delivered on a yearly basis subject to certain reporting requirements. As of December 31, 2020, 58,090 bonus shares had been issued.

## UNIVERSITY OF MIAMI

**Exclusive License Agreement**

In September 2019, we entered into a global royalty-bearing, exclusive license to all data, know-how, inventions, and technology generated by University of Miami, Emory University, and Massachusetts General Hospital in a Phase 2 clinical trial of arimoclomol in ALS with the A4V SOD1 mutation. Orphazyme paid USD 75K cash up-front and agreed to future payments of certain license fees, a milestone upon regulatory approval in ALS, and annual fees as well as a low single-digit royalty on net sales of licensed products or processes on a product-by-product and country-by-country basis. Annual fees will be creditable against royalty and milestone payments.

## CYTRX

**Asset Purchase Agreement**

In May 2011, Orphazyme entered into an Asset Purchase Agreement with the biopharmaceutical company CytRx. Pursuant to this agreement, CytRx irrevocably sold and transferred certain preclinical and clinical data, patents, and other intellectual property rights, and other assets, including contractual rights and obligations relating to a portfolio of chemical compounds, including arimoclomol, to Orphazyme. Under the terms of the agreement, Orphazyme paid USD 150K cash up-front and agreed to make future payments to CytRx contingent upon achievement of specified clinical, regulatory, and sales milestones and royalties on a specified percentage of net sales of products containing one of the compounds purchased.

The maximum aggregate amount of milestone payments that may be triggered for non-ALS or stroke products is $12.1 million for the first product (arimoclomol) and $10.3 million for the second product developed. For ALS or stroke products, the maximum aggregate amount of milestone payments per product is $23.8 million. Further details about our material agreements are available in our filings with the SEC.

# CORPORATE GOVERNANCE

In order to maintain the trust of the Company's stakeholders, Orphazyme is committed to ensuring transparent and good corporate governance. As a Danish company listed on Nasdaq Copenhagen, Orphazyme is subject to the Danish Recommendations on Corporate Governance.

The Recommendations on Corporate Governance are best practice guidelines for the management of companies admitted to trading on a regulated market.

Orphazyme intends to comply with the Recommendations on Corporate Governance in all material respects, however, due to the current size of the Company and the nature of its present operations, the company has opted to deviate from the recommendations in the following areas:

• Orphazyme has decided to only publish annual reports and half-yearly financial reports;
• the annual report does not include information on individual board members' participation in board meetings;
• the general conclusion of the latest evaluation of the Board of Directors is not described in the annual report, but is accounted for by the Chairman at the annual general meeting and share-based remuneration is offered to the Board of Directors and have a maturity of one year from the date of allocation.

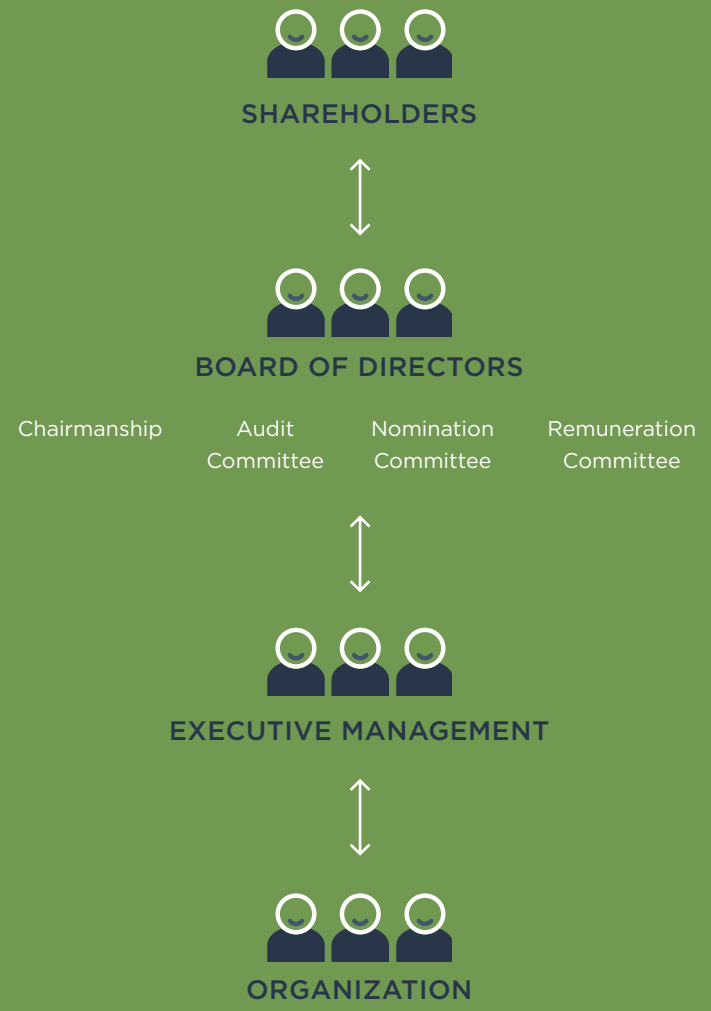
Orphazyme's corporate governance statement includes a summary of the Company's governance structure, a description of internal control and financial reporting procedures, Orphazyme's position on the Recommendation on Corporate Governance as well as a complete list of the Company's comments to recommendations that the Company opted to deviate from.

The corporate governance statement is available on www.orphazyme.com

## Corporate Governance Statement

### BOARD OF DIRECTORS

The Board of Directors is responsible for the overall and strategic management and proper organization of Orphazyme's business and operations and it supervises the Company's activities, management, and organization. The Board of Directors



## BOARD OF DIRECTORS

| Name | Position | Independent[1] | Year of first appointment | Expiration of term |
|---|---|---|---|---|
| Georges Gemayel | Chairman | Independent | 2012 | 2021 |
| Bo Jesper Hansen | Deputy Chairman | Independent | 2010 | 2021 |
| Martin Bonde | Member | Independent | 2010 | 2021 |
| Sten Verland | Member | Independent | 2010 | 2021 |
| Rémi Droller | Member | Independent | 2015 | 2021 |
| Martijn Kleijwegt | Member | Independent | 2017 | 2021 |
| Catherine Moukheibir | Member | Independent | 2017 | 2021 |
| Anders Hedegaard | Member | Independent | 2017 | 2021 |
| Carrolee Barlow | Member | Independent | 2020 | 2021 |

[1] The Company has based its assessment of independence on the basis of criteria set out in the current Corporate Governance Recommendations (as defined below).

appoints and dismisses the members of the Executive Management, who are responsible for the day-to-day management of the Company.

### MEETINGS

The Board of Directors normally holds at least five regular meetings annually, including a strategy review, plus ad-hoc meetings as required. Extraordinary board meetings are convened by the Chairman when necessary or when requested by a member of the Board of Directors, a member of the Executive Management, or by the Company's auditor. The Board of Directors forms a quorum when more than half of its members are represented, including the Chairman or the Deputy Chairman. Resolutions of the Board of Directors are passed by a simple majority of the votes present at the meeting. In the event of equal votes, the Chairman or, in his absence, the Deputy Chairman shall have the casting vote. The Board of Directors conducts an annual evaluation of the effectiveness, performance, achievements, and competencies of the Board of Directors and of the individual members as well as the collaboration with the Executive Management.

The members of the Board of Directors elected by the general meeting are elected for a term of one year. Members of the Board of Directors may be re-elected.

### BOARD COMMITTEES

To support the Board of Directors in its duties, the Board of Directors has established and appointed an Audit Committee, a Nomination Committee, and a Remuneration Committee. These committees are charged with reviewing issues pertaining to their respective fields that are due to be considered at board meetings. The Board of Directors has decided to establish a Science Committee with effect following the annual general meeting 2021.

### Committee Charters

Visit Orphazyme.com for more information and to view written charters.

### INTERNAL CONTROLS AND FINANCIAL REPORTING PROCEDURES

As a foreign private issuer with American Depositary Shares listed on the Nasdaq Global Select Market, we are required to comply with the Sarbanes-Oxley Act. The Board of Directors, the Audit Committee, and the Executive Management are responsible for risk management and internal controls into its financial reporting and approve general policies in that regard. The Audit Committee assists the Board of Directors in overseeing the reporting process and the most important risks involved in this respect. The Executive Management is responsible for the effectiveness of the internal controls and risk management and for the implementation of such controls aimed at mitigating the risk associated with the financial reporting. Orphazyme has internal control and financial reporting procedures aimed at enabling it to monitor its performance, operations, funding, and risk.

The Board and Executive Management assess risks on an on-going basis, including risks related to financial reporting, and assess measures to manage, reduce, or eliminate identified risks. The Audit Committee reviews selected key risk areas on a frequent basis, including significant accounting estimates and material changes to accounting policies. At least once a year, the Audit Committee oversees a review of current internal controls to determine whether they are effective in relation to the risks identified in the financial reporting process.

Orphazyme has adopted and defined an internal control framework that identifies key processes, inherent risks, and control procedures in order to secure appropriate accounting processes. The control procedures include a variety of processes in order to prevent any misrepresentation, significant errors, omissions, or fraudulent behaviour. The control procedures are tested on a regular basis and reported to the Audit Committee.

Orphazyme's independent auditors are appointed for a term of one year by the shareholders at the Company's annual general meeting upon recommendation from the Audit Committee. The Board of Directors assesses the independence and competencies and other matters pertaining to the auditors. The framework for the auditors' compensation and duties, including audit and non-audit tasks, is agreed annually between the Board of Directors and the auditors based on recommendations from the Audit Committee.

## AUDIT COMMITTEE

The Audit Committee reviews accounting and audit matters that by decision of the Board of Directors or the Audit Committee require a more thorough evaluation and assess the internal controls and risk management systems of Orphazyme. Its duties also include supervision of the Company's auditors and review of the audit process. In addition, the Chairwoman of the Audit Committee also monitors our Whistleblower Hotline.

In accordance with the Recommendations on Corporate Governance of the Danish Committee on Corporate Governance issued in November 2017 (the "Corporate Governance Recommendations"), the Company has decided that the Chairman of the Board of Directors may not also be the Chairman of the Audit Committee and that the members of the Audit Committee are required to meet the independence requirements set out in the Corporate Governance Recommendations.

In addition, at least one member shall have accounting or audit qualifications and between them, the members shall possess such expertise and experience as to provide an updated insight into, and experience in, the financial, accounting, and audit aspects of companies with shares admitted to trading and official listing on a regulated market.

The Audit Committee shall consist of no less than three members appointed by and among the Board of Directors, including the Chairman of the Audit Committee. The members of the Audit Committee meet the independence requirement set out in the Corporate Governance Recommendations. The Chief Executive Officer and/or the Chief Financial Officer and the Company's external auditor shall participate in meetings of the Audit Committee if requested by the Audit Committee and the external auditor shall attend at least one meeting per year or the relevant part hereof where the Executive Management is not present.

## NOMINATION COMMITTEE

The Nomination Committee shall assist the Board of Directors with ensuring that appropriate plans and processes are in place for the nomination of candidates to the Board of Directors and the board committees. Moreover, the Nomination Committee shall evaluate the composition of the Board of Directors. This includes making recommendations for nomination or appointment of members of (a) the Board of Directors and (b) the board committees established by the Board of Directors.

The Nomination Committee consists of no less than three members appointed by and among the Board of Directors. The members of the Nomination Committee meet the independence requirements set out in the Corporate Governance Recommendations.

## REMUNERATION COMMITTEE

The Remuneration Committee ensures that the Company maintains a Remuneration Policy for the members of the Board of Directors and the Executive Management, and to evaluate and make recommendations for the remuneration of the members of the Board of Directors and the Executive Management. The Remuneration Committee shall annually evaluate the composition of the Executive Management. This includes making recommendations for nomination or appointment of members of the Executive Management. Moreover, the Remuneration Committee shall assist the Board of Directors with ensuring that appropriate plans and processes are in place for nomination of candidates to the Executive Management.

The Remuneration Committee shall consist of no less than three members appointed by and among the Board of Directors. The members of the Remuneration Committee meet the independence requirements set out in the Corporate Governance Recommendations.

**COMMITTEE COMPOSITION**

| Name | Audit | Remuneration | Nomination |
|---|---|---|---|
| Georges Gemayel | | | Chairperson |
| Bo Jesper Hansen | | Chairperson | |
| Catherine Moukheibir | Chairperson | | |
| Anders Hedegaard | | Member | |
| Martijn Kleijwegt | Member | | |
| Martin Bonde | | | Member |
| Rémi Droller | | Member | |
| Sten Verland | Member | | Member |
| Carrolee Barlow | | | |

Chairperson = Chairperson  Member = Member

# CORPORATE SOCIAL RESPONSIBILITY

## OUR BUSINESS

Orphazyme is a late-stage biopharmaceutical company pioneering the Heat-Shock Protein (HSP) response for rare, neurodegenerative diseases. We are harnessing the amplification of Heat-Shock Proteins, or HSPs, in order to develop and commercialize novel therapeutics aimed at addressing unmet medical needs in underserved diseases.

Our product candidate, arimoclomol, is a first-in-class Heat-Shock Protein amplifier, in development for the treatment of Niemann-Pick disease Type C (NPC), Inclusion Body Myositis (IBM), Amyotrophic Lateral Sclerosis (ALS), and Gaucher disease.

We are establishing our own commercial organization to bring our products to market, if approved, and use external suppliers for manufacturing and supply.

Our headquarters is in Copenhagen and we have subsidiaries in the U.S. and Switzerland focused on facilitating our interactions with key stakeholders including patients, healthcare providers, and payors.

## OUR RESPONSIBILITY

At Orphazyme, we are working towards a common vision: To profoundly impact the lives of patients with neurodegenerative orphan diseases and their families.

Our team is connected by a set of core values focused on care, courage, perseverance, and integrity. These core values underpin our corporate culture and guide our responsibility towards society, patients, employees, and our stakeholders.

Operating always with the highest ethical standards, we strive to demonstrate respect for key moral principles and comply with international regulations and good practice guidelines. As most of our business activities are outsourced, we work closely with our partners to ensure compliance with these regulations and guidelines. Below we have described Orphazyme's most significant Corporate Social Responsibility (CSR) risks and how we handle them.

## OUR CORE VALUES

### COURAGE

To do what others have not. People living with diseases like NPC and ALS have waited long enough for someone to stand beside them in their fight.

### INTEGRITY

Going the extra mile for those who need it. We share the energy, passion, and vision to make a meaningful difference for the rare disease community.

### CARE

So that every day can be better than the last. The treatments we develop are just the start of our efforts to help people living with rare, orphan diseases.

### PERSEVERANCE

Because bravery deserves company. People living with orphan diseases, their families, and their loved ones do not let odds get in their way. Neither do we.

# CSR REPORTING AREAS

## HUMAN RIGHTS

Orphazyme acknowledges and supports the maintenance of internationally declared human rights and bases its work on the UN Universal Declaration of Human Rights and the interpretation that it is the responsibility of the State to protect, and the companies' responsibility to respect, these rights.

Orphazyme conducts its business in accordance with the ethical and scientific principles governing clinical research on human subjects, as set out in the Declaration of Helsinki and the International Conference on Harmonization (ICH) guidelines on Good Clinical Practice (GCP).

Orphazyme interprets human rights to comprise respect for diversity. The company promotes a diverse and inclusive workplace and has a diversity policy to increase the diversity among members of the Board of Directors of the company and other management levels pursuant to section 139a of the Danish Companies Act and the Danish Recommendations on Corporate Governance. The Board of Directors' target is to include at least two female board members by the end of 2021. In 2020, Orphazyme expanded its Board of Directors to consist of nine members, which is now comprised of two women and seven men. Our diversity policy further outlines our key goals. Furthermore, the company has a whistleblower policy in place to allow reporting of potential violations of the respect for diversity in the workplace.

Orphazyme conducts business in a highly regulated industry. The company has assessed its risks related to human rights as being limited. However, Orphazyme will continue to support and respect internationally declared human rights and does not and will not employ child labor.

**Training of employees in relation to whistleblower policy**

## ANTI-CORRUPTION & BRIBERY

Orphazyme is committed to maintaining the highest standards of conduct and will not tolerate the use of bribery or corruption to achieve its business objectives. Our policies on bribery and corruption are clearly set out in our anti-corruption policy and our employee handbook and are reinforced annually at mandatory meetings and during training.

Employees must decline any expensive gifts, money, trips, or other such offerings from business contacts. This also includes receiving services from suppliers without paying for them.

In 2021, Orphazyme expects to expand its commitments in relation to anti-corruption and bribery by adopting a specific policy on gifts and entertainment to ensure ongoing compliance with anti-corruption & bribery regulation within this area.

## RISK ANALYSIS

Summary of estimated risk for key environmental and social factors:

### VERY LIMITED

**Environment and climate**

**Reason:** modest number of employees; use of external suppliers

### LIMITED

**Social and employees**

**Human rights**


**Anti-corruption**

**Reason:** business operates in a strictly regulated environment

# CSR REPORTING AREAS

Orphazyme has a whistleblower policy in order to allow reporting of potential violations of laws and serious violations of internal policies and procedures, including fraud and anti-corruption.

In 2021, Orphazyme will assess its whistleblower scheme in order to ensure compliance with enhanced whistleblower regulation as a consequence of EU Directive 2019/1937.

- **No bribery and corruption violations identified or reported in 2020**
- **Training of employees in relation to anti-corruption & bribery**

## ENVIRONMENT & CLIMATE

Orphazyme acknowledges the challenges associated with climate change.

The company conducts its business in a highly regulated industry and climate and follows applicable rules on hazardous substances. However, considering the business of the company, Orphazyme's general potential impact on the environment and climate is viewed as minimal. As such, specific environment and climate policies have not been developed at this time. Furthermore, Orphazyme keeps a record of all accidents and have no records of spill of hazardous substances. The company has a highly educated staff that follows established procedures both during use and at disposal of hazardous substances. As such, use of hazardous substances is connected with a very low and controlled risk.

However, despite our very limited impact, we continue to focus on efficient management of office materials and may update our travel policies, based on our learnings from working virtually during COVID-19, to further reduce our impact.

## OUR PEOPLE AND EMPLOYEE-WELL BEING

A diverse, skilled, and healthy workforce is crucial to the success of Orphazyme and our ability to serve patients and the rare disease community. The health and safety of the employees is a high priority and Orphazyme continually works to ensure that all systems and processes live up to international standards for laboratories. All employees working in the laboratories are trained in the systems, processes and mandatory and ongoing education in relation to workplace safety.

Orphazyme conducts mandatory Health and Safety surveys (APVs) on a regular basis to assess the working environment at the company. The surveys address several aspects of the working environment such as psychological, ergonomics, and chemical working environment.

Orphazyme offers health insurance to its employees.

We value diversity in gender, age, ethnicity, nationality, religion, education, sexual orientation, work history, opinions, and skills at all levels of our business. Our recruitment process is focused on balancing representation in our teams. Currently, our staff consists of 65% females and 35% males, including 57% female and 43% male employees at director level or above. Further information on employee ratios can be found on page 29.

We operate an inclusive workplace and are committed to ensuring an environment that is free of discrimination, harassment, and bullying. Our Workplace Assessment Survey is an important tool in identifying any issues and in monitoring employee satisfaction.

## COVID-19

During 2020, the COVID-19 pandemic has put additional pressure on physical and mental health caused by health risks and lockdowns. The Company has put great focus on ensuring the health and well-being of our employees during this time by introducing guidelines and instructions to those working at home or needing to come into the workplace and by providing personal protective equipment. These, along with other measures, seek to ensure the physical and psychological health of our employees in connection with the pandemic and working from home.

We continuously monitor the pandemic and its potential impact on our business and the health and well-being of our employees.

**WE ARE FOCUSED ON SUPPORTING THE UN SUSTAINABLE DEVELOPMENT GOALS AND HAVE IDENTIFIED SIX OF THESE GOALS WHERE WE SEE OUR WORK ADDING MOST VALUE**

# CSR REPORTING AREAS

## BUSINESS ETHICS

To ensure that business is conducted ethically, fairly, and with integrity, Orphazyme has adopted a Conflict of Interest Policy. Orphazyme is committed to conducting business in a manner that ensures that no Orphazyme representatives are influenced by undue personal interests.

In case of a potential conflict of interest Orphazyme representatives must receive approval and guidance from his or her supervisor and Legal Affairs.

Orphazyme has also adopted a Code of Business Conduct and Ethics, which contains guidelines which the company's directors, officers, and employees are expected to adhere to in the conduct of the Company's business.

Orphazyme will continue its efforts in training its employees in conducting business ethically, fairly, and with integrity.

**Training of employees in business ethics and conflict of interest.**

## OUR COMMITMENT

We value personal development and provide opportunities for our employees to participate in professional events and training courses aimed at strengthening core competencies.

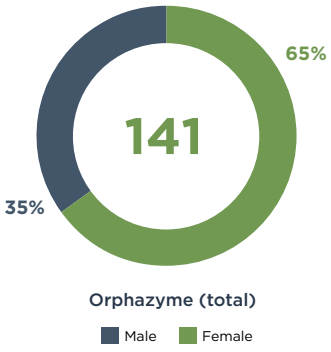
We promote the health of our employees and have a variety of initiatives in place focused on general well-being, including all-staff town-hall meetings to foster employee engagement, flexible working options, and "take-a-breather" sessions, focused on mindfulness.

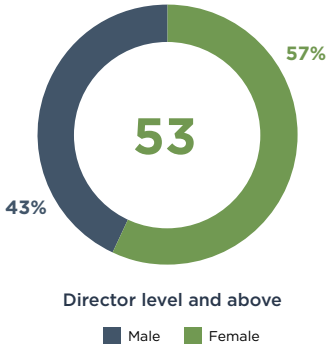
Access our Governance Documents

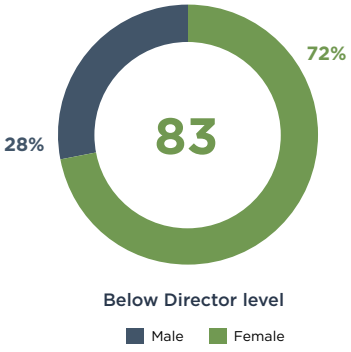
## PROGRESS IN 2020

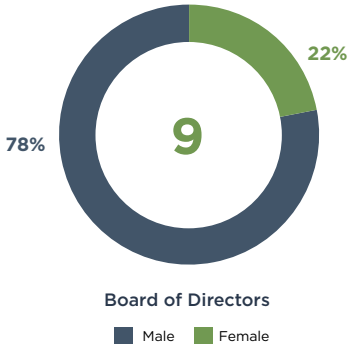
**In 2020, the company upgraded its legal risk and compliance system. All employees are trained in Company policies using our risk and compliance system. As part of the onboarding of new employees, they receive training in the policies of the company.**

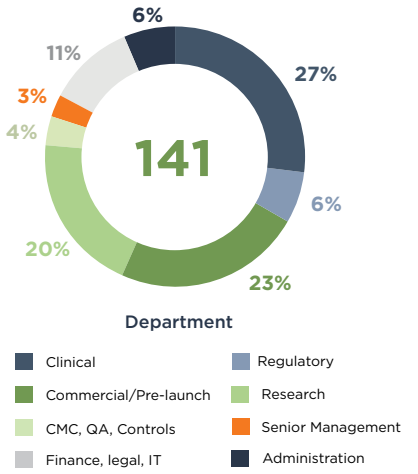
## EMPLOYEE NUMBER AND GENDER RATIOS BY LEVEL



# RISK MANAGEMENT

## RISKS THAT THREATEN THE ACHIEVEMENT OF OUR KEY OBJECTIVES

**Key objective**
To successfully commercialize our product candidate in key markets.

**Risks that threaten the achievement of our key objectives**
Commercial risks include, but are not limited to: Our ability to obtain and maintain orphan designation/status, which will provide us with marketing exclusivity; our ability to establish our own commercialization capabilities, including sales and marketing expertise in core markets; our ability to partner with third parties for distribution and/or commercialization; competition from other companies developing treatments for similar diseases, which could render our products obsolete or limit our ability to generate revenues; our ability to gain sufficient payor coverage and reimbursement; market acceptance by physicians & payors and issues with product manufacturing, including third-party CMOs, availability, and supply.

**Our actions to mitigate the risks**
In anticipation of potential regulatory authorizations, we have accelerated our pre-commercialization and launch activities and assess risks on an ongoing basis. Our commercial structure and operations are currently focused on NPC and on building an organization comprised of highly qualified talent with experience in the oversight and execution of product launches and commercial enterprises in the rare disease space. Our management team has a highly successful track record of launching and commercializing rare disease products across the United States and international markets.

**Key objective**
To successfully conduct and complete clinical trials of arimoclomol and gain regulatory approvals required for commercialization.

**Risks that threaten the achievement of our key objectives**
Designing and conducting clinical trials is complex, costly, and time-consuming and neither the results nor timing can be predicted with any certainty. Our clinical trials have been and may continue to be affected by the Covid-19 pandemic, and the CRO's who help conduct our trials, may also be impacted. There is a risk that results may not confirm previous results, or will produce adverse or inconclusive results; the FDA or other regulatory authorities may not consider the endpoints of our clinical trials to predict or provide clinically meaningful results or demonstrate sufficient evidence to gain requisite regulatory approvals. Furthermore additional clinical trials or data may be required to obtain such approvals, resulting in increased costs, significant delays to filing with the authorities, filing a narrower indication than anticipated or the abandonment of efforts to commercialize one or more of the Company's product candidates.

**Our actions to mitigate the risks**
We make every effort to design and plan our clinical trials in the most diligent manner and perform them under strict regulatory guidelines. Professional organizations (CROs) run our trials and we conduct inspections and internal quality audits to maximize quality, safety and efficacy. We frequently interact with regulatory authorities to ensure we are advancing our programs in the most appropriate and expedient manner.

**Key objective**
To use our expertise, including proprietary know-how, to select and develop new molecular entities (NMEs) for other rare, neurodegenerative diseases.

**Risks that threaten the achievement of our key objectives**
To a large extent, our success depends on our ability to obtain and maintain patents and other intellectual property rights for our products. Our IP is the basis for our current products and any potential new leads, and thus any threats to our IP rights could be detrimental to our future pipeline of product candidates.

**Our actions to mitigate the risks**
We are developing a suite of new molecular entities (NMEs) and have attracted highly talented resources to continue to develop and explore new leads. In addition, we are consistently monitoring our IP in order to not only protect our rights and minimize legal claims, but also strengthen our rights and current technology platform. We believe that our patent portfolio has a wide scope of protection and geographical coverage.

*In addition to risks threatening the achievement of our key objectives, we are exposed to pervasive risks that threaten our business.*

## PERVASIVE RISKS THAT THREATEN OUR BUSINESS

**Pervasive risk**
Lack of sufficient financing.

**How the risk threatens our business**
We have not received approval for any product candidate for commercial sale and, as a result, have incurred significant financial losses, and may continue to incur significant financial losses in the future, which makes it difficult to assess the future viability of the Company. Furthermore, in order to execute our strategy, we may need to raise additional capital in the future and such funding may not be available on favorable terms. Failure to generate or secure sufficient financial resources could have a material adverse effect on the Company's business and/or prospects.

**Our actions to mitigate the risks**
In 2020, we raised additional capital through a directed share issue and private placement (February) and a successful global offering and IPO in the US (September), which further strengthened our balance sheet. Our existing cash, together with potential revenue from monetization of a Priority Review Voucher and income from arimoclomol, if approved, is expected to be sufficient to support our operations into 2022. Further details on our Outlook can be found on page 9. Our financial situation and risks are assessed on an ongoing basis and reported to the Audit Committee and the Board of Directors.

**Pervasive risk**
Non-compliance with legislation and industry standards.

**How the risk threatens our business**
We are subject to regulatory and legislative obligations in order to conduct business. These requirements are subject to change and if we do not remain abreast of the regulations and actively work to comply, we are at risk of either losing or not obtaining required approvals or we may receive penalties, fines, or suspension of our approvals or registrations. There is also a risk that cybersecurity attacks could compromise data privacy or cause interruption to our operations. Our business operations and current and future relationships with healthcare professionals, principal investigators, consultants, customers and third-party payors in the United States and elsewhere may be subject, directly or indirectly, to applicable anti-kickback, fraud and abuse, false claims, physician payment transparency, health information privacy and security and other healthcare laws and regulations, which could expose us to substantial penalties.

**Our actions to mitigate the risks**
Our organization has increased regulatory resources to facilitate interactions with regulators, actively monitor the current regulatory environment and ensure our compliance. We have implemented new IT-security procedures in order to reduce the risk of cybercrime. We have internal training requirements for all employees and contracting external suppliers, we ensure they have adequate measures in place to comply with relevant regulatory requirements.

**Pervasive risk**
Attraction and retention of talent.

**How the risk threatens our business**
The success of our company depends on our ability to attract, integrate, manage, and retain qualified personnel or key employees. Failure to do so could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects . The market for qualified personnel is competitive and the Company may not succeed in recruiting personnel to, for instance, commercialize its products as currently envisaged, or it may fail to effectively replace current personnel who depart with qualified or effective successors. Our employees may not be able to come to work as a result of COVID-19.

**Our actions to mitigate the risks**
We believe we have established an attractive workplace at Orphazyme, underpinned by high ethical standards. We are committed to maintaining a working environment that is diverse, free of discrimination, harassment, and bullying. We have established initiatives that provide opportunities for personal and professional development, improved health-wellbeing, work-life flexibility, and a participation in the overall success of Orphazyme through our long-term incentive plans.

# SHAREHOLDER INFORMATION

## OWNERSHIP

Orphazyme is dual-listed on Nasdaq Copenhagen (since November 16, 2017) under the ticker symbol ORPHA and on Nasdaq Global Select Market in the US (since September 29, 2020) under the ticker symbol ORPH.

We conduct our communication in accordance with the applicable rules and regulations set forth by the Danish Financial Supervisory Authority, the U.S. Securities and Exchange Commission ("SEC"), and each of the exchanges on which we are listed. As of December 31, 2020, the number of registered Shareholders totalled 14,599 shareholders holding a total of 27,817,294 shares, representing 80% of the total share capital of 34,697,703. As of December 31, 2020, 522,143 shares were represented by American Depositary Shares (ADS). In February 2021, there was a capital increase of 170,131 ordinary shares related to the issue of Matching Shares and the issue of bonus shares to Kansas Life Sciences Development Company, Inc., resulting in a total share capital of 34,948,387 as of March 2, 2021, the date of this annual report.

All shareholders have the same rights, including in respect of eligibility to receive dividends and participate in share buybacks, governed under Danish law. ADS holders will not have shareholder rights and may not be able to exercise their right to vote the shares underlying the ADSs. ADS holders have the contractual rights of an ADS holder, as provided in the deposit agreement among the Company, the depositary and holders and beneficial owners of ADSs from time to time. As a result, ADS holders may only exercise voting rights with respect to the shares underlying the ADSs in accordance with the provisions of the deposit agreement, Further information about ADS holder rights can be found at https://orphazyme.gcs-web.com/ads-faqs and in our filings with the SEC.

Orphazyme has not declared or made any dividend payments for the last two financial years. Currently, the Company intends to use all available financial resources as well as revenue, if any, for purposes of the Company's current and future business. As of the date hereof, the Company does not expect to make dividend payments within the foreseeable future.

**18%**

**ownership by major shareholders**

**Dual-listed**

**Nasdaq Copenhagen, Denmark**
**Nasdaq Global Select, US**

## MAJOR SHAREHOLDERS

| Major shareholder | Company address | Share capital % |
|---|---|---|
| LSP V Coöperatieve U.A. | Johannes Vermeer, Plein 9, 1071 DV Amsterdam, Netherlands | 7.8%[1] |
| Sunstone Life Science Ventures Fund II K/S | Store Strandstræde 18A, st., 1255 København K | 5.2% |
| Coöperatieve Aescap Venture I U.A. | Barbara Strozzilaan 101, 1083 HN Amsterdam, Netherlands | 5.1% |

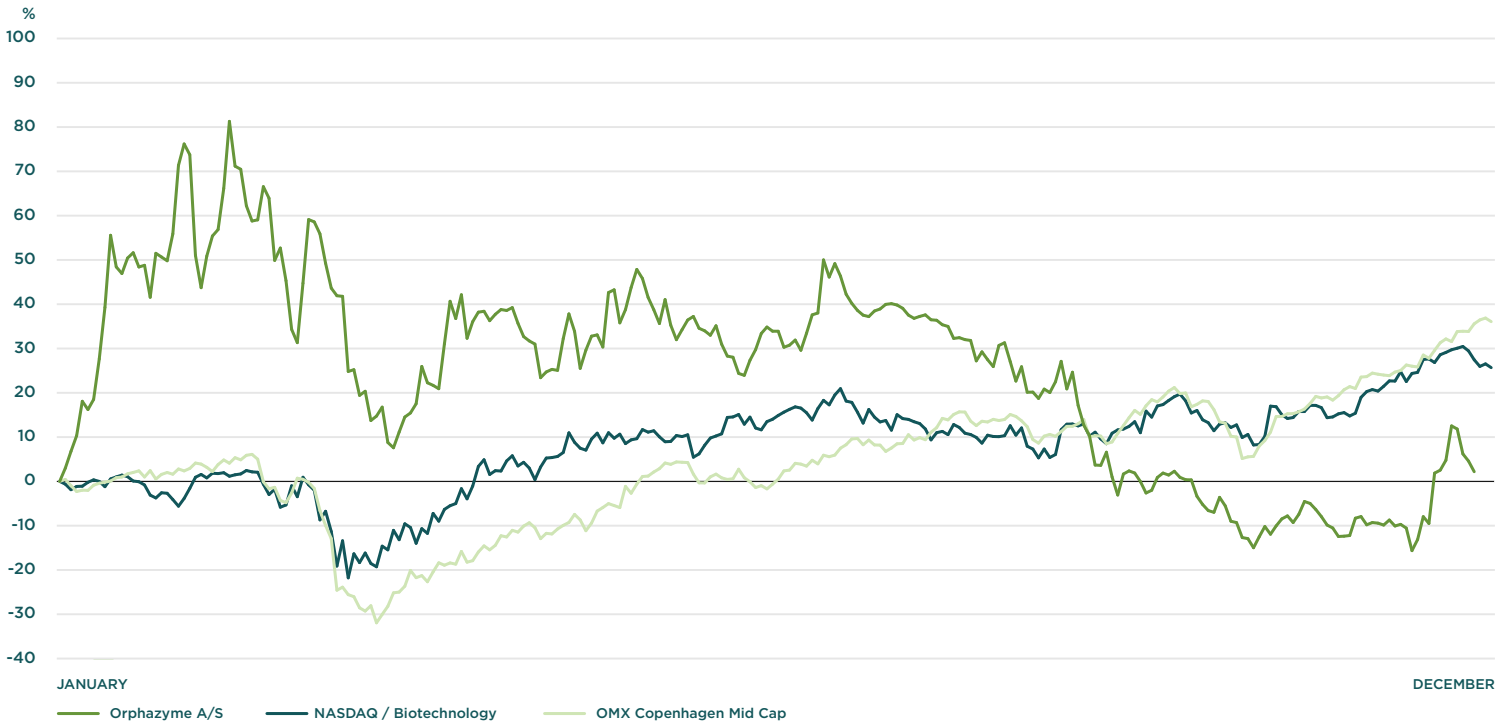
[1] Orpha Pooling B.V. is an investment vehicle 88.2% owned by LSP V Coöperatieve U.A. and 11.8% owned by ALS Invest 2 B.V. LSP Management B.V. is the director of LSP V Coöperatieve U.A and the director of Orpha Pooling B.V. and exercises voting rights on behalf of Orpha Pooling B.V.

## INVESTOR RELATIONS

Orphazyme's Investor Relations' primary goal is to ensure relevant, accurate, and timely communication of information to our stakeholders. We provide both company announcements (containing potentially share-sensitive information) and investor news (pertaining to interesting news that is deemed not share-sensitive) through the appropriate channels.

Additionally, we file annual and half-year reports, host analyst and investor calls/ meetings and maintain an informative and transparent website where visitors can find relevant materials such as announcements, financial reports, SEC filings, corporate documents, company policies, etc. Orphazyme maintains an active dialogue with the sell-side and is currently followed by six analysts, as highlighted below. A list of our covering analysts is also available via our Investor Relations website.

## SHARE PERFORMANCE COMPARISON 2020 (DECEMBER 31, 2019 = 0%)




## COVERING ANALYSTS

**USA**
Bank of America - Tazeen Ahmad
Cowen - Ritu Baral
Guggenheim - Yatin Suneja

**Europe**
Danske Equities - Thomas Bowers
Oddo BHF - Martial Descoutures
Redeye - Anders Hedlund

## FINANCIAL CALENDAR

**Annual General Meeting:** Thursday, March 25, 2021
**Interim Report H1 2021:** Tuesday, August 24, 2021

## QUESTIONS? PLEASE CONTACT:

**Anders Vadsholt, Chief Financial Officer**
afv@orphazyme.com

# BOARD OF DIRECTORS

## GEORGES GEMAYEL

Chairman of the Board

**Member since:** 2012 (Chairman, 2014)
**Born in:** 1960
**Nationality:** American
**Committees:** Nomination (Chairman)

**Special competencies:**
Dr Gemayel has significant management and executive experience from the global pharmaceutical industry. He holds a Master's and PhD in Pharmacology from Paris-Sud University and a Docteur d'Exercice en Pharmacie, St. Joseph University.

**Current positions:**
Chairman of the Board of Dynacure, Enterome SA, and OxThera AB, and a member of the Board of Directors of Supernus Pharmaceuticals Inc. (publ).

## BO JESPER HANSEN

Deputy Chairman of the Board

**Member since:** 2010 (Deputy Chairman, 2017)
**Born in:** 1958
**Nationality:** Danish
**Committees:** Remuneration (Chairman)

**Special competencies:**
Dr Hansen has extensive experience in orphan drugs, both from the operations and supervisory point of view and has broad and current know-how of the biotechnology environment. He holds an MD and a PhD in Medicine from the University of Copenhagen.

**Current positions:**
Chairman of the Board of Laborie Inc., Innoventa Medica ApS, Karo Pharma AB; member of the Board of Ascelia Pharma AB and Reapplix A/S. Venture Partner at Wellington Partners; Advisory Consultant for Aescap 2.0, Nordic Capital, EQT AB and Broad Street Principal Investments Europe Ltd. & senior business advisor for HBM Ventures Ltd.

## CATHERINE MOUKHEIBIR

**Member since:** 2017
**Born in:** 1959
**Nationality:** American, Lebanese, British
**Committees:** Audit (Chairman)

**Special competencies:**
Ms Moukheibir has in-depth experience in the pharmaceutical and banking industries and a successful track-record in leading Audit Commitees of publicly-traded companies. She holds a Master's degree in Economics and an MBA, both from Yale.

**Current positions:**
Non-Executive Board Member and Chair of the Audit Committee at CMR Surgical. Member of the Board of Directors of Ironwood Pharmaceuticals, Inc. and Kymab.

# BOARD OF DIRECTORS

## RÉMI DROLLER

**Member since:** 2015
**Born in:** 1975
**Nationality:** French
**Committees:** Remuneration

**Special competencies:**
Mr Droller has extensive experience as a biotechnology investor and a proven track-record in negotiating several successful transactions. He holds a Master's in Molecular Biology from Université Pierre et Marie Curie and a Master's in Finance and Management of Innovation from AgroParisTech.

**Current positions:**
Managing Partner of Kurma Partners SA and member of the Board of Directors of Dynacure SAS, ImCheck Therapeutics SAS, OxThera AB, AM Pharma BV, Flamingo Therapeutics BV, Vico Therapeutics BV, and Pharvaris BV.

## STEN VERLAND

**Member since:** 2010
**Born in:** 1957
**Nationality:** Danish
**Committees:** Nomination, Audit

**Special competencies:**
Dr Verland is a serial entrepreneur in biotechnology companies and has extensive investment and managerial experience. He holds a Master's in Biology and Mathematics and a PhD in Immunology from the University of Copenhagen.

**Current positions:**
Co-Founder and Board Member at Sunstone Capital A/S, Board Member and General Partner at Sunstone Life Science Ventures A/S, Board Member of STipe Therapeutics ApS, Anergis SA, MinervaX ApS, OxThera AB, the Danish VC and PE Association (DVCA), Board Member and Executive Management in certain Sunstone Group companies; member of the Executive Management at Verland Capital ApS, Verland Holding ApS, Verland Holding II ApS, and Genobiotix ApS.

## MARTIJN KLEIJWEGT

**Member since:** 2017
**Born in:** 1955
**Nationality:** Dutch
**Committees:** Audit

**Special competencies:**
Mr Kleijwegt has extensive experience as a major European venture-capital investor as well as in-depth experience from the pharmaceutical industry. He holds a Master's degree in Economics from the University of Amsterdam.

**Current positions:**
Founder and Managing Partner at LSP Management Group BV and a member of the Board of Directors of AM Pharma BV, Kiadis Pharma N.V. (publ), OxThera AB, Eloxx Pharmaceuticals Ltd., Arvelle Therapeutics BV, Vico Therapeutics Holding BV, and Pharvaris BV.

# BOARD OF DIRECTORS

## ANDERS HEDEGAARD

**Member since:** 2017
**Born in:** 1960
**Nationality:** Danish
**Committees:** Remuneration

**Special competencies:**
Mr Hedegaard has extensive knowledge of the healthcare industry, both in product development and commercialization. Master of Science in Chemical Engineerng and Biochemistry from the Technical University of Denmark.

**Current positions:**
Anders Hedegaard is currently Chief Executive Officer of Rodenstock Group and Chairman of the Board of Directors of ALK-Abelló A/S.

## CARROLEE BARLOW

**Member since:** 2020
**Born in:** 1963
**Nationality:** American
**Committees:** None

**Special competencies:**
Carrolee holds a PhD in Molecular and Development Biology from the Karolinska Medical Nobel Institution, an MD from the University of Utah School of Medicine and a BA in English from the University of Utah.

**Current positions:**
Chief Medical Officer of E-Scape Bio, Inc. since January 2019. In the past five years, Dr Barlow has served as Chief Executive Officer and Member of the Board of Directors of the Parkinson's Institute and Clinical Center. Dr Barlow is currently a member of the Board of Directors of Supernus Pharmaceuticals and is a member of the scientific and/or clinical advisory boards of Neurametrix Inc., Kainos Medicine and the Silverstein Foundation.

## MARTIN BONDE

**Member since:** 2010
**Born in:** 1963
**Nationality:** Danish
**Committees:** Nomination

**Special competencies:**
Dr Bonde has executive experience and in-depth knowledge of the biotechnology environment in the Nordic countries. He holds a Graduate Diploma in Business Administration from Copenhagen Business School, an MSc. and a PhD in Chemical Engineering from the Technical University of Denmark.

**Current positions:**
Chief Executive Officer of Inthera Bioscience AG; member of the Board of Directors of BioInnovation Institute Fonden, BII Holdings A/S, Visiopharm A/S, Chief Executive Officer of Bohrs Tower ApS as well as a member of the Board of Directors and the Executive Management of Biotopix ApS.

# MANAGEMENT

## EXECUTIVE MANAGEMENT

### ANDERS VADSHOLT

**Interim Chief Executive Officer and Chief Financial Officer**

Anders joined Orphazyme in May 2016 as Chief Financial Officer. He has served as Interim Chief Executive Officer since December 2020.

Anders has 20+ years' experience from biotech and corporate finance. Previously at Topotarget, BankInvest Biomedical Venture, 7TM Pharma, and Carnegie.

Anders is currently a member of the Board of Directors at OxThera AB and Owner of Alpha Healthcare Investments ApS.

Anders holds a BSc in Corporate Law from the University of Aalborg, an MBA in Finance and Strategy from the University of Melbourne, and an MSc in Corporate Law and Economics from Copenhagen Business School.

**Born in:** 1969
**Nationality:** Danish

## KEY EMPLOYEES

### THOMAS KIRKEGAARD JENSEN, PHD

**Chief Scientific Officer, Co-Founder**

Thomas joined the Company as Co-Founder and Chief Executive Officer in 2009 and became Chief Scientific Officer in March 2010.

Thomas is currently a member of the Executive Management of Dare to Dream ApS, an expert reviewer for the European Research Council and a member of the Advisory Board for the Rare Disease Report.

Thomas holds a BSc in Biochemistry, an MSc in Human Biology and a PhD in Medicine from the University of Copenhagen.

**Born in:** 1977
**Nationality:** Danish

### THOMAS BLAETTLER, MD

**Chief Medical Officer**

Thomas joined Orphazyme in November 2016 as Chief Medical Officer.

Thomas has 13+ years' experience in neuroscience development. Previously at Roche, Bristol-Myers Squibb, and Novartis.

Thomas Blaettler holds a Doctorate in Medicine from the University of Zürich and a Medical School Certificate Swiss State Examination from the Medical School of the University of Zürich. He is a board-certified neurologist by the Swiss Medical Association (the Foederation Medicorum Helveticorum).

**Born in:** 1967
**Nationality:** Swiss and Danish

## PRESIDENT ORPHAZYME US, INC

### MOLLY PAINTER

**President, US**

Molly joined Orphazyme in January 2020 as President, Orphazyme US, Inc.

Molly brings 20+ years' general management experience at leading global pharmaceutical and biotech companies including J&J, Baxalta, Shire, and most recently Takeda.

Molly has dedicated a significant portion of her career focused on rare disease.

Molly is currently on the Board of Directors of the Healthcare Businesswomen's Association.

Molly has a BSc in Communication from Miami University and an MBA from Northwestern University Kellogg School of Management.

**Born in:** 1976
**Nationality:** American

# GLOSSARY

**Amyotrophic Lateral Sclerosis (ALS)**
Also called Lou Gehrig's disease; a rare neuromuscular disease, which is rapidly progressive and fatal, usually within two to five years.

**Breakthrough Therapy Designation (BTD)**
FDA designation that is intended to expedite the development and review of drugs to treat serious and life-threatening diseases in cases where preliminary evidence shows that the drug may provide substantial improvements over available therapy.

**Early Access Program (EAP)**
Provides patients with serious, life-threatening diseases or conditions with unmet medical needs access to investigational drugs before they are approved.

**European Medicines Agency (EMA)**
Regulatory agency in Europe that facilitates development and access to medicines, evaluates applications for marketing authorization and monitors the safety of medicines.

**Fast-Track Designation**
FDA designation that is intended to facilitate the development and expedite review of drugs for serious diseases with an unmet medical need, getting new drugs to patients earlier.

**Gaucher disease**
An inherited metabolic disorder caused by mutations in a protein called glucocerebrosidase, which leads to the accumulation of certain sugar-containing lipids.

**Heat-Shock Proteins**
Heat-Shock Proteins (HSPs) are molecular chaperones constituting a natural system that makes other proteins work correctly and guard against toxicity arising from misfolded proteins and dysfunctional cellular recycling systems.

**Inclusion Body Myositis (IBM)**
An acquired, rare, and slowly-progressing protein-aggregation disease.

**Marketing Authorization Application (MAA)**
A submission to apply for marketing approval for a drug from EMA.

**New Drug Application (NDA)**
A submission to apply for marketing approval for a drug from the FDA.

**Niemann-Pick disease Type C (NPC)**
A rare, inherited, progressive, and often fatal neurodegenerative disease.

**Orphan Drug Designation**
This program provides orphan status to drugs and biologics, which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases.

**Rare Pediatric Disease Designation**
The Rare Pediatric Disease Designation is granted by the FDA to drugs that have a potential to treat rare pediatric diseases. Orphazyme has received such a designation for arimoclomol for NPC. The designation entails the potential receipt of a so-called Priority Voucher upon marketing authorization, if certain criteria are met. The voucher can be redeemed to provide Priority Review of a subsequent NDA for a different product.

**US Food and Drug Administration (FDA)**
US regulatory agency responsible for ensuring the safety, efficacy and security of human and veterinary drugs, biological products and medical devices.

# CORPORATE INFORMATION

## COMMERCIAL BANKERS

**Danske Bank**
Holmens Kanal 2-12
DK-1092 Copenhagen K

**Nordea**
Vesterbrogade 8
DK-1620 Copenhagen

**BNY Mellon**
240 Greenwich St
New York, NY 10286
USA

## LEGAL COUNSEL

**Gorrissen Federspiel, Advokatpartnerselskab**
Axeltorv 2
DK-1609 Copenhagen V

**Cooley LLP**
55 Hudson Yards
New York, NY 10001
USA

## INDEPENDENT AUDITORS

**EY Godkendt Revisionspartnerselskab**
Dirch Passers Allé 36
DK-2000 Frederiksberg

**Annual report**
This annual report will be available on www.orphazyme.com and printed copies are available upon request.

**Annual General Meeting**
The Annual General Meeting will be held on March 25, 2021 at 5.00 PM CET at:
COBIS, Ole Maaløes Vej 3,
DK-2200 Copenhagen N

**Disclaimer**
This annual report may contain certain forward-looking statements, including in respect of the anticipated commercialization of arimoclomol. Although the Company believes its expectations are based on reasonable assumptions, all statements other than statements of historical fact included in this company announcement about future events are subject to (i) change without notice and (ii) factors beyond the Company's control. These statements may include, without limitation, any statements preceded by, followed by, or including words such as "target," "believe," "expect," "aim," "intend," "may," "anticipate," "estimate," "plan," "project," "will," "can have," "likely," "should," "would," "could", and other words and terms of similar meaning or the negative thereof. Forward-looking statements are subject to inherent risks and uncertainties beyond the Company's control that could cause the Company's actual results, performance, or achievements to be materially different from the expected results, performance, or achievements expressed or implied by such forward-looking statements, including the risk that applicable regulatory authorities fail to approve arimoclomol on the anticipated timeline or at all. Except as required by law, the Company assumes no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in the forward-looking statements, even if new information becomes available in the future.

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# CONSOLIDATED STATEMENTS OF PROFIT OR LOSS AND OTHER COMPREHENSIVE INCOME

**For the years ended December 31**

| (DKK 000), EXCEPT PER SHARE AND SHARE DATA | Note | 2020 | 2019 | 2018 |
|---|---|---|---|---|
| Research and development expenses | 2.1, 2.2 | (361,284) | (285,413) | (196,525) |
| General and administrative expenses | 2.3 | (247,250) | (50,541) | (35,127) |
| **Operating loss** | | **(608,534)** | **(335,954)** | **(231,652)** |
| Financial income | 2.6 | 2,444 | 316 | 5 |
| Financial expenses | 2.6 | (29,071) | (7,359) | (3,453) |
| **Loss before tax** | | **(635,161)** | **(342,997)** | **(235,100)** |
| Income tax benefit | 2.7 | 1,915 | 5,500 | 5,500 |
| **Net loss for the year** | | **(633,246)** | **(337,497)** | **(229,600)** |
| *Items that will be reclassified subsequently to the Statement of Profit or Loss:* | | | | |
| Exchange difference from translation of foreign operations net of tax DKK 0 | | 605 | 67 | 42 |
| **Total comprehensive loss** | | **(632,641)** | **(337,430)** | **(229,558)** |
| Weighted-average shares outstanding | | 28,366,469 | 20,024,692 | 20,008,827 |
| Loss per share, basic and diluted | 4.3 | (22.32) | (16.87) | (11.49) |

The accompanying notes form an integral part of these consolidated financial statements.

# CONSOLIDATED STATEMENTS OF FINANCIAL POSITION

**As of December 31**

| ASSETS (DKK 000) | Note | 2020 | 2019 |
|---|---|---|---|
| **Non-current assets** | | | |
| Intangible assets | 3.1 | 12,454 | 10,539 |
| Right-of-use assets | 3.2 | 14,859 | 13,903 |
| Property, plant, and equipment | 3.3 | 4,687 | 3,685 |
| Corporation tax receivable | 2.7 | 2,750 | 2,750 |
| Deferred tax assets | 2.7 | 2,065 | - |
| Prepayments and deposits | 3.4 | 2,014 | 1,652 |
| **Total non-currents assets** | | **38,829** | **32,529** |
| **Current assets** | | | |
| Corporation tax receivable | 2.7 | 5,500 | 5,500 |
| Prepayments and other receivables | 3.4 | 51,235 | 19,137 |
| Cash | 3.7 | 726,929 | 123,588 |
| **Total current assets** | | **783,664** | **148,225** |
| **Total assets** | | **822,493** | **180,754** |

| EQUITY AND LIABILITIES (DKK 000) | Note | 2020 | 2019 |
|---|---|---|---|
| **Equity** | | | |
| Share capital | 4.2 | 34,698 | 19,984 |
| Share premium | 4.2 | 2,082,254 | 924,021 |
| Other reserves | | 6,494 | 7,982 |
| Accumulated deficit | | (1,502,921) | (899,018) |
| **Total equity** | | **620,525** | **52,969** |
| **Non-current liabilities** | | | |
| Borrowings | 3.6 | 23,830 | 51,606 |
| Lease liabilities | 3.2 | 9,877 | 9,813 |
| Other non-current liabilities | 3.6 | 1,634 | 378 |
| **Total non-current liabilities** | | **35,341** | **61,797** |
| **Current liabilities** | | | |
| Current borrowings | 3.6 | 33,349 | 12,813 |
| Current lease liabilities | 3.2 | 3,657 | 2,876 |
| Trade payables and accruals | 3.6 | 72,135 | 32,390 |
| Tax payables | | 4,159 | - |
| Other liabilities | 3.6 | 53,327 | 17,909 |
| **Total current liabilities** | | **166,627** | **65,988** |
| **Total equity and liabilities** | | **822,493** | **180,754** |

The accompanying notes form an integral part of these consolidated financial statements.

# CONSOLIDATED STATEMENTS OF CHANGES IN SHAREHOLDERS' EQUITY

| | | | | Other reserves | | | |
|---|---|---|---|---|---|---|---|
| (DKK 000) | Notes | Share capital | Share premium | Foreign currency translation reserve | Share-based compensation – acquisition of intangible assets | Accumulated deficit | Total |
| **Balance as of December 31, 2017** | | 19,928 | 924,021 | 0 | 9,972 | (338,219) | 615,702 |
| Net loss for the year | | | | | | (229,600) | (229,600) |
| Other comprehensive income (loss) | | | | 42 | | - | 42 |
| Total other comprehensive income (loss) | | | | 42 | - | (229,600) | 229,600) |
| Transactions with owners: | | | | | | | |
| Capital increase in connection with issuance of bonus shares | 3.1 | 11 | | | (902) | 891 | - |
| Share-based compensation expense | 2.5 | - | | | | 2,105 | 2,105 |
| Total transactions with owners | | 11 | - | - | (902) | 2,996 | 2,105 |
| **Balance as of December 31, 2018** | | 19,939 | 924,021 | 42 | 9,070 | (564,823) | 388,249 |
| Net loss for the year | | | | | | (337,497) | (337,497) |
| Other comprehensive income (loss) | | | | 67 | | - | 67 |
| Total other comprehensive income (loss) | | | | 67 | - | (337,497) | (337,430 |
| Transactions with owners: | | | | | | | |
| Capital increase in connection with issuance of bonus shares | 3.1 | 26 | | | (1,197) | 1,171 | - |
| Issuance of Matching Shares, net of costs | 2.5 | 19 | | | | | 19 |
| Share-based compensation expense | 2.5 | - | | | | 2,131 | 2,131 |
| Total transactions with owners | | 45 | - | - | (1,197) | 3,302 | 2,150 |
| **Balance as of December 31, 2019** | | **19,984** | **924,021** | **109** | **7,873** | **(899,018)** | **52,969** |
| Net loss for the year | | | | | | (633,246) | (633,246) |
| Other comprehensive income (loss) | | | | 605 | | | 605 |
| Total other comprehensive income | | | | 605 | - | (631,317) | (632,641) |
| Transactions with owners: | | | | | | | - |
| Capital increase in connection with issuance of bonus shares | 3.1 | 21 | | | (2,094) | 2,073 | - |
| Capital increase in connection with exercise of RSUs | 4.2 | 13 | 717 | | | | 730 |
| Capital increase in connection with private placement | 4.2 | 7,033 | 738,458 | | | | 745,491 |
| Transaction costs in connection with private placement | | | (51,243) | | | | (51,243) |
| Capital increase in connection with U.S. listing | 4.2 | 7,616 | 526,918 | | | | 534,534 |
| Transaction costs in connection with U.S. listing | | | (56,616) | | | | (56,616) |
| Issuance of Matching Shares, net of costs | 2.5 | 31 | | | | | 31 |
| Share-based compensation expense | 2.5 | | | | | 27,270 | 27,270 |
| Total transactions with owners | | 14,714 | 1,158,234 | - | (2,094) | 29,343 | 1,200,19 |
| **Balance as of December 31, 2020** | | **34,698** | **2,082,254** | **714** | **5,780** | **(1,502,921)** | **620,525** |



The accompanying notes form an integral part of these consolidated financial statements.

# CONSOLIDATED STATEMENTS OF CASH FLOWS

| FOR THE YEARS ENDED DECEMBER 31, (DKK 000) | Note | 2020 | 2019 | 2018 |
|---|---|---|---|---|
| **Net loss** | | (633,246) | (337,497) | (229,600) |
| Reversal of non-cash items: | | | | |
| Equity-settled share-based compensation expense | 2.5 | 28,105 | 2,549 | 2,105 |
| Depreciation and amortization | 3.1, 3.2, 3.3 | 5,200 | 3,803 | 1,366 |
| Financial income | 2.6 | (2,444) | (316) | (5) |
| Financial expenses | 2.6 | 29,071 | 7,359 | 3,453 |
| Income tax benefit | 2.7 | (1,915) | (5,500) | (5,500) |
| Exchange rate adjustments | | - | - | (491) |
| Change in working capital: | | | | |
| Change in prepayments, deposits, and other receivables | 3.4 | (33,662) | 4,920 | (14,578) |
| Change in trade payables, accruals, and other liabilities | 3.6 | 76,424 | (2,844) | 5,943 |
| Corporation taxes received | 2.7 | 5,500 | 5,500 | 5,500 |
| Corporation taxes paid | 2.7 | (1,431) | - | - |
| Interest received | | 45 | 388 | 5 |
| Interest paid | | (10,723) | (5,181) | (2,962) |
| **Net cash used in operating activities** | | **(539,076)** | **(326,818)** | **(234,764)** |
| **Investing activities** | | | | |
| Purchase of intangible assets | 3.1 | (2,736) | (508) | (1,603) |
| Purchase of property, plant, and equipment | 3.3 | (2,365) | (2,777) | (743) |
| **Net cash used in investing activities** | | **(5,101)** | **(3,285)** | **(2,346)** |
| **Financing activities** | | | | |
| Proceeds from borrowings | 3.6 | - | 62,758 | - |
| Repayment of borrowings | | (10,535) | - | - |
| Repayment of lease obligations | 3.2 | (2,970) | (3,838) | - |
| Proceeds from issuance of shares | 2.5 | 1,280,786 | 19 | - |
| Transaction costs related to issuance of shares | | (107,859) | - | - |
| **Net cash provided by financing activities** | | **1,159,422** | **58,939** | **-** |
| **Net change in cash** | | 615,245 | (271,164) | (237,110) |
| Effects of changes in exchange rates | | (11,904) | 46 | 81 |
| Cash at the beginning of the year | | 123,588 | 394,706 | 631,735 |
| **Cash at the end of the year** | | **726,929** | **123,588** | **394,706** |

The accompanying notes form an integral part of these consolidated financial statements.

SECTION 1

# BASIS OF PREPARATION AND SIGNIFICANT ACCOUNTING POLICIES

Section 1 provides a summary of the significant accounting policies applied by the Group, Management's key accounting estimates and judgements, and new IFRS standards applicable to the Group. A detailed description of accounting policies and key accounting estimates and judgements related to specific financial statement line items is presented in each note to the relevant line item.

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## 1.1 CORPORATE INFORMATION

Orphazyme A/S (the "Company") is a late-stage biopharmaceutical company harnessing the amplification of Heat Shock Proteins, or HSPs, in order to develop and commercialize novel therapeutics for the treatment of neurodegenerative orphan diseases. The Company is headquartered in Copenhagen, Denmark and is publicly traded on Nasdaq Copenhagen. In September 2020, the Company listed American Depositary Shares (ADSs) on the Nasdaq Global Select Market and raised gross proceeds of USD 83.7 million (DKK 534.5 million).

In April 2018, a wholly-owned subsidiary, Orphazyme U.S., Inc., was incorporated in Delaware, USA and in March 2020, a wholly-owned subsidiary, Orphazyme Schweiz GmbH, was incorporated in Zug, Switzerland (together with Orphazyme A/S, "Orphazyme" or "the Group"). By establishing local subsidiaries, the Company aims to directly support the U.S. and European markets and establish closer relationships with the medical, patient, and financial communities as Orphazyme expands its development programs and global reach.

These consolidated financial statements were approved and authorized for issuance by the Board of Directors on March 2, 2021.

**Information on COVID-19**

Our business, operations and clinical studies were, of course, impacted by the effects of COVID-19. Although our clinical studies continued without interruption during 2020, there weredelays and increased total costs arising from the implications of COVID-19; namely, increased home nursing costs and direct-to-patient shipments of our drug product for both our clinical studies as well as our early access program in the U.S. However, we have not recognized any write-offs, impairments of assets, or losses due to onerous contracts.

The COVID-19 pandemic is also having an effect on other aspects of our business, including: our third-party manufacturers, and other third parties; albeit with no material effect or impact. The COVID-19 pandemic may, in the long-term, affect the productivity of our staff; our ability to attract, integrate, manage and retain qualified personnel or key employees; our global supply chains and relationships with vendors and other parties; significant disruption of global financial markets; and reduced ability to secure additional funding. We continuously monitor the COVID-19 pandemic and its potential impact on our business and financials.

## 1.2 BASIS OF PREPARATION

The consolidated financial statements of the Group have been prepared in accordance with International Financial Reporting Standards (IFRS) as issued by the International Accounting Standards Board (IASB) and in accordance with IFRS as endorsed by the EU and further requirements in the Danish Financial Statements Act. All entities in the Orphazyme Group follow the same Group accounting policies.

The consolidated financial statements have been prepared on a going concern basis and are presented in Danish Kroner, or DKK, which is both the functional and presentation currency of the Company. The functional currency of Orphazyme US, Inc. is the US dollar (USD) and the functional currency of Orphazyme Schweiz GmbH is the Swiss Franc (CHF). Where indicated, amounts are rounded to the nearest thousand.

**Materiality**
The consolidated financial statements are prepared based on the concept of materiality, which considers both quantitative and qualitative factors. Items that are considered individually significant or are required under the minimum presentation requirements of IFRS are presented separately. If items are individually immaterial, they are aggregated with other items of similar nature in the financial statements or in the notes.

## 1.3 SIGNIFICANT ACCOUNTING POLICIES

A detailed description of accounting policies and significant accounting estimates and judgements related to specific financial statement line items is presented in each note to the relevant line item. The consolidated financial statements have been prepared on a historical cost basis except for share-based compensation and the embedded derivative in our borrowings, which is measured at fair value.

**Principles of consolidation**
The consolidated financial statements of the Group include the financial statements of the parent company, Orphazyme A/S (the "Parent Company"), Orphazyme US, Inc. and Orphazyme Schweiz GmbH, fully-owned subsidiaries over which the Parent Company has control. A company controls an entity when the company (i) is exposed to, or has rights to, variable returns from its involvement with the entity, (ii) has power over the entity (i.e. existing rights that give it the current ability to direct the activities of the entity), and (iii) has the ability to use its power to affect the returns of the entity. The Parent Company reassesses whether it controls an entity if facts and circumstances indicate that there are changes to one or more of the three elements of control. Consolidation of an entity begins when the Parent Company obtains control and ceases when the Parent Company has lost control of the entity. On consolidation, intercompany income and expenses, intercompany receivables, and payables, and unrealized gains and losses on transactions between the consolidated companies are eliminated.

**Translation of foreign currencies**
Items included in the financial statements of each of the Orphazyme entities are measured using the currency of the primary economic environment in which the entity operates, or functional currency. On initial recognition, transactions denominated in foreign currencies are recorded using the foreign exchange spot rate at the transaction date. For monetary assets and liabilities, differences arising between the foreign exchange spot rates at the transaction date and the date of settlement or period-end exchange rates are recognized in the Statement of Profit or Loss as financial income or financial expenses. On consolidation, the assets and liabilities of Orphazyme US, Inc. and Orphazyme Schweiz GmbH are translated from the subsidiary's functional currency to DKK at the exchange rate in effect at the balance sheet date and the Statement of Profit or Loss and Other Comprehensive Income is translated from the subsidiary's functional currency to DKK at the date of the underlying transaction or average exchange rate of the period if there are no significant fluctuations in exchange rate throughout the period. The exchange rate differences arising on translation for consolidation are recognized in other comprehensive income (loss).

## 1.3 SIGNIFICANT ACCOUNTING POLICIES (CONTINUED)

**Statement of cash flows**
The statement of cash flows is presented using the indirect method and shows cash flows resulting from operating activities, investing activities, financing activities, and the Group's cash at the beginning and end of the year, including any effects of exchange rate changes.

*Cash flows used in operating activities* converts items in the Statement of Profit or Loss from the accrual basis of accounting to the cash basis of accounting. Non-cash items such as foreign exchange gains and losses, depreciation, amortization, and changes in working capital are reversed from the net loss for the year and actual cash receipts and payments are included.

*Cash flows from investing activities* shows payments related primarily to the purchase of licenses and property, plant, and equipment.

*Cash flows from financing activities* shows proceeds from share issuance, borrowings, net of transaction costs, repayment of debt, and lease payments.

**Segment information**
Although Orphazyme established a US subsidiary in 2018 and a Swiss subsidiary in 2020, the Group is managed and operated as one business unit that is reflected in the internal reporting. No separate lines of business or separate business entities have been identified with respect to any product candidate or geographical market and no segment information is currently disclosed in the Group's internal reporting. For the years ended December 31, 2020, 2019, and 2018, the Group generated no revenue and all material non-current assets are located in Denmark.

## 1.4 SIGNIFICANT ACCOUNTING ESTIMATES AND JUDGEMENTS

The use of reasonable estimates and judgements is an essential part of the preparation of the consolidated financial statements. Given the uncertainties inherent in the Group's business activities, Management must make certain significant accounting estimates and judgements, which affect the application of accounting policies and therefore the reported amounts of assets, liabilities, revenue, expenses, and disclosures in the consolidated financial statements. The significant accounting estimates and judgements identified are those that have a significant risk of resulting in a material adjustment to the consolidated financial statements. Management bases its estimates on historical experience, assumptions, and information currently available and deemed to be reasonable at the time the consolidated financial statements are prepared. However, actual amounts may differ from the estimated amounts as more detailed information becomes available. Estimates and assumptions are reviewed on an ongoing basis and, if necessary, changes are recognized in the period in which the estimate is revised. Management has made significant accounting estimates and judgements in the following areas, which are further presented in each note to the relevant financial statement line items:

- Estimate of research and development expenses associated with clinical trials (Note 2.2) and related prepayments (Note 3.4) and accruals (Note 3.6)
- Estimate of inputs and assumptions used in share-based compensation valuation models (Note 2.5)
- Estimate of the fair value of licenses (Note 3.1)
- Estimate relating to the incremental borrowing rate to measure lease liabilities (Note 3.2)
- Judgement and estimate relating to pre-launch drug product inventory (Note 3.5)
- Judgement regarding the recognition of deferred tax assets related to taxable losses to be carried forward (Note 2.7)
- Judgement regarding management's assessment of the company's ability to continue as a going concern (Note 4.1)

Please refer to the specific referenced notes for further information on the significant accounting estimates and judgements as well as assumptions applied.

## 1.5 NEW IFRS STANDARDS APPLICABLE TO THE GROUP

The Group applied for the first-time certain standards and amendments which are effective for annual periods beginning on or after January 1, 2020. The Group has not early adopted any other standard, interpretation or amendment that has been issued but is not yet effective.

*Amendments to IFRS 3 Definition of a business*

The amendments to IFRS 3 Business Combinations clarifies that to be considered a business, an integrated set of activities and assets must include, at a minimum, an input and a substantive process that, together, significantly contribute to the ability to create output. Furthermore, it clarifies that a business can exist without including all of the inputs and processes needed to create outputs. These amendments had no impact on the consolidated financial statements of the Group, but may impact future periods should the Group enter into any business combinations.

*Amendments to IFRS 7, IFRS 9 and IAS 39 Interest Rate Benchmark Reform*

The amendments to IFRS7, IFRS 9 and IAS 39 Financial Instruments: Recognition and Measurement provide a number of reliefs, which apply to all hedging relationships that are directly affected by interest rate benchmark reform. A hedging relationship is affected if the reform gives rise to uncertainty about the timing and/or amount of benchmark-based cash flows of the hedged item or the hedging instrument. These amendments have no impact on the consolidated financial statements of the Group as it does not have any interest rate hedge relationships.

*Amendments to IAS 1 and IAS 8 Definition of Material*

The amendments provide a new definition of material that states, "information is material if omitting, misstating or obscuring it could reasonably be expected to influence decisions that the primary users of general purpose financial statements make on the basis of those financial statements, which provide financial information about a specific reporting entity." The amendments clarify that materiality will depend on the nature or magnitude of information, either individually or in combination with other information, in the context of the financial statements. A misstatement of information is material if it could reasonably be expected to influence decisions made by the primary users. These amendments had no impact on the consolidated financial statements of the Group.

*Conceptual Framework for Financial Reporting issued on 29 March 2018*

The Conceptual Framework is not a standard, and none of the concepts contained therein override the concepts or requirements in any standard. The purpose of the Conceptual Framework is to assist the IASB in developing standards, to help preparers develop consistent accounting policies where there is no applicable standard in place and to assist all parties to understand and interpret the standards. This will affect those entities which developed their accounting policies based on the Conceptual Framework. The revised Conceptual Framework includes some new concepts, updated definitions and recognition criteria for assets and liabilities and clarifies some important concepts. These amendments had no impact on the consolidated financial statements of the Group.

*Amendments to IFRS 16 Covid-19 Related Rent Concessions*

On May 28, 2020, the IASB issued Covid-19-Related Rent Concessions - amendment to IFRS 16 Leases. The amendments provide relief to lessees from applying IFRS 16 guidance on lease modification accounting for rent concessions arising as a direct consequence of the Covid-19 pandemic. As a practical expedient, a lessee may elect not to assess whether a Covid-19 related rent concession from a lessor is a lease modification. A lessee that makes this election accounts for any change in lease payments resulting from the Covid-19 related rent concession the same way it would account for the change under IFRS 16, if the change were not a lease modification. The amendment applies to annual reporting periods beginning on or after 1 June 2020. Earlier application is permitted. This amendment had no impact on the consolidated financial statements of the Group.

*New IFRS standards not yet effective*

The IASB has issued a number of new standards and updated some existing standards, which are effective for accounting periods beginning January 1, 2021 or later. Therefore, they are not incorporated in these consolidated financial statements. There are no standards presently known that are not yet effective and that would be expected to have a material impact on our current or future reporting periods.

SECTION 2

# RESULT OF THE YEAR

**Section 2 presents details related to Orphazyme's Statement of Profit or Loss and Other Comprehensive Income, including Research and Development expenses and General and Administrative expenses, Government Grants, Employee costs, and Share-based Compensation costs. The Group does not yet generate revenue. In addition, this section comprises Financial Income, Financial Expenses, and Income Taxes.**

**In this section**


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## 2.1 RESEARCH AND DEVELOPMENT EXPENSES

### § ACCOUNTING POLICIES

Research expenses comprise of costs incurred during the very early stages of the drug development cycle from initial drug discovery until the drug is ready for administration to humans. The activities initially focus on identifying a single drug candidate with a profile that will support a decision to initiate development activities. Before selection of the final drug candidate, it is tested in animals to gather efficacy, toxicity and pharmacokinetic information.

Development expenses comprise costs incurred during the different phases of clinical drug development starting in phase 1, when the drug is administered to humans for the first time, through phases 2 and 3, and subsequent activities to obtain marketing authorizations, which will permit Orphazyme to eventually market and sell the drug products.

In line with industry practice, Orphazyme expenses all research costs. Development costs that do not meet the definition of an asset are also expensed as incurred. Due to regulatory and other uncertainties inherent in the development of new products, development costs do not qualify for capitalization as intangible assets until marketing approval by a regulatory authority is obtained or highly probable. In addition, pre-launch inventory costs are recognized under Research and Development (see Note 3.5).

Clinical trial costs are a significant component of research and development expenses. The Company's clinical trials are performed by third-party Contract Research Organizations (CROs) and in order to estimate the amount of costs to charge to expense Management has developed expense models for each clinical trial based on estimates and assumptions.

The clinical trials generally have three distinctive stages.

- Start-up stage: initial setting up of the trial
- Treatment stage: site and trial management during the dosing period
- Wrap-up stage: close down and reporting of the trial

For each clinical trial for which information about the actual services delivered by the CRO are not provided on a regular current basis, the Company reviews the approved budgets for the clinical trial from the original executed agreements and categorizes the individual costs according to the three stages described above. The start-up activities, which include site recruitment, regulatory applications and investigator meetings, usually are performed reasonably uniformly throughout the start-up stage and the related costs are expensed ratably over this stage, which reflects the manner in which related services are rendered by the CRO.

## 2.1 RESEARCH AND DEVELOPMENT EXPENSES (CONTINUED)

The start-up stage is followed by the treatment stage, during which patients are dosed with the drug under study and results are monitored and measured. The costs incurred in this stage of the trial, which comprises the major portion of the total cost of the clinical trial, is mainly driven by the number of enrolled patients undergoing treatment. The Company estimates the costs attributable to activities performed in this stage of the trial on a per-patient basis. These costs are expensed over the treatment stage as patients are enrolled and undergo treatment, as reported by the CRO. After the last patient has been treated, the trial begins to be closed down and activities are performed related to data quality assurance and analysis. These activities are performed reasonably uniformly throughout the wrap-up stage and are expensed ratably over this last stage. Other costs, such as central laboratory costs and drug supply costs, are expensed as incurred, which is typically when the service has been rendered or the goods delivered.

CROs invoice the Company upon the occurrence of predetermined milestones (such as the enrollment of patients); however, the timing of these invoices and the Company's related payments often do not correspond directly to the level of performance of contracted activities. To the extent payments are made by the Company in advance of the related activities performed by the CROs, they are included in prepayments to vendors (see Note 3.4) and expensed in accordance with the expense model discussed above. To the extent that the payments are made by the Company following the performance of the related activities, the expense is reflected as an accrual (see Note 3.6) in accordance with the expense model.

Research and development expenses include costs arising from research and clinical development activities including employee costs for research and development personnel (i.e. salaries, bonuses, employer contributions to pension schemes, share-based compensation), legal expenses related to the protection, defense and enforcement of the Company's intellectual property, as well as depreciation of right-of-use assets associated with facilities and equipment used for research and development purposes. The following table presents research and development expenses recognized for the years ended December 31:

External costs comprise mainly expenses related to third party vendors providing services related to our research and development activities and facility costs. External costs in 2020 include expense from write-down of pre-launch inventory of DKK 12.4m (Note 3.5).

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| External costs | 261,136 | 218,143 | 154,952 |
| Employee costs (Note 2.4) | 96,108 | 64,167 | 40,281 |
| Depreciation and amortization (Notes 3.1, 3.2, 3.3) | 4,040 | 3,103 | 1,292 |
| **Total research and development expenses** | **361,284** | **285,413** | **196,525** |

**Estimate of research and development expenses associated with clinical trials**
Accounting for clinical trial costs related to activities performed by Contract Research Organizations (CROs) and other external vendors requires Management to make significant estimates regarding the timing of the expense recognition of these costs. The diverse nature of services being provided by CROs, the different compensation arrangements that exist for each type of service, and the limitation in the availability of information related to when certain clinical activities are performed add complexity to the estimation of the timing of expense recognition for services rendered by CROs and other vendors in connection with clinical trials. In addition, the COVID-19 pandemic has increased the estimation uncertainty of clinical trial costs, in particular the timing of the expense recognition due to potential delays in services being performed.

## 2.2 GOVERNMENT GRANTS

### § ACCOUNTING POLICIES

Government grants are recognized when there is reasonable assurance that the funding will be received, and all underlying conditions will be fulfilled. Income from grants is recognized in the Statement of Profit or Loss as a reduction of the related expenses being reimbursed in the period when the related expenses are incurred.

Government grants comprise research funding from the Danish government and the European Union. The grants received by Orphazyme provide reimbursement for certain project-specific research and development expenses, including wages and salaries. During the year ended December 31, 2020, Orphazyme has received DKK 0.0 million (2019: DKK 0.1 million; 2018: DKK 2.1 million) in government grant funding, which was receivable as of December 31, 2019.

As of the year ended December 31, 2020, the total amount still receivable under these grants is DKK 0.1 million (2019: DKK 0.4 million) and is classified as Current Other Receivables in the Statement of Financial Position, as all remaining funding from grants is receivable within the next year (Note 3.4). One grant has been paid to Orphazyme in advance and income in the amount of DKK 0.0 million (2019: DKK 0.1 million) related to this grant has been deferred and presented in the Statement of Financial Position as current other liabilities (Note 3.6). All the grants received are subject to repayment clauses upon breach of conditions to maintain the terms under which the grant was awarded. Orphazyme has complied with and anticipates continuing to fully comply with all such terms.

## 2.3 GENERAL AND ADMINISTRATIVE EXPENSES

### § ACCOUNTING POLICIES

General and administrative expenses include salaries for administrative employees and Executive Management, remuneration to the Board of Directors, share-based compensation costs, depreciation of right-of-use assets associated with facilities not used for research and development purposes, and investor relations. In addition, we include pre-commercial activities in general and administrative expenses, such as the preparation of an Early Access Program for NPC, tradename costs, market and pricing studies and related costs.

The following table presents general and administrative expenses for the years ended December 31:

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| External costs | 118,971 | 23,847 | 19,250 |
| Employee costs (Note 2.4) | 127,120 | 25,995 | 15,803 |
| Depreciation (Notes 3.2 and 3.3) | 1,159 | 699 | 74 |
| **Total general and administrative expenses** | **247,250** | **50,541** | **35,127** |

External costs comprise expenses related to third party vendors providing assistance with establishing a commercial organization and the escalation of launch preparation activities, including hiring a commercial team in our subsidiaries in the U.S. and Switzerland, market access activities, and medical affairs activities to further engage with the scientific community through communication and education programs. In addition, external costs comprise expenses related to administrative services such as legal and accounting support, IT, and investor relations.

## 2.4 EMPLOYEE COSTS

### § ACCOUNTING POLICIES

Employee costs primarily comprise salaries, bonuses, social security contributions, share-based compensation, vacation and sick leave as well as the employer portion of pension contributions. In addition, severance payments or termination benefits are also included under Employee Costs. The cost of these benefits is recognized as an expense as services are received. All employee pension plans are defined contribution plans and not defined benefit plans.

Employees are eligible to receive a discretionary bonus subject to certain predefined and individual goals as determined by the Board of Directors. Employees are also eligible to receive an extraordinary bonus at the discretion of the Board of Directors.

The following table presents Employee Costs, including remuneration to the Board of Directors and Executive Management, for the years ended December 31, 2020, 2019, and 2018. Refer to note 4.5 for more discussion on remuneration of Board of Directors and Executive Management.

| EMPLOYEE COSTS (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| Salaries | 131,606 | 68,719 | 42,243 |
| Cash bonus | 40,481 | 8,707 | 4,583 |
| Share-based compensation (Note 2.5) | 27,258 | 2,405 | 2,145 |
| Pensions | 11,313 | 5,561 | 3,058 |
| Other social security contributions | 5,172 | 875 | 326 |
| Other staff costs | 3,083 | 862 | 966 |
| **Total employee costs excluding board remuneration** | **218,913** | **87,129** | **53,321** |
| Board remuneration | 3,469 | 2,888 | 2,763 |
| Board share-based compensation (Note 2.5) | 846 | 145 | - |
| **Total employee costs** | **223,228** | **90,162** | **56,084** |
| **Recognized as follows in the Statement of Profit or Loss:** | | | |
| Research and development expenses | 96,108 | 64,167 | 40,281 |
| General and administrative expenses | 127,120 | 25,995 | 15,803 |
| **Total employee costs** | **223,228** | **90,162** | **56,084** |
| Average number of full-time employees | 117 | 74 | 46 |
| Year-end number of full-time employees | 141 | 86 | 57 |

## 2.5 SHARE-BASED COMPENSATION COSTS

### § ACCOUNTING POLICIES

**Equity-settled awards**

Shares awarded under the long-term incentive program ("LTIP") are equity-settled awards. The fair value of these awards is determined at the date of grant, resulting in a fixed fair value at grant date that is not adjusted for future changes in the fair value of the awards that may occur over the service period. The fair value of the LTIP awards has been determined using the Monte-Carlo model. Further details of the valuation models are presented below.

The fair value of equity-settled awards with service conditions and non-market performance conditions is recognized as compensation expense pro rata over the service period to the extent such awards are estimated to vest. The compensation expense is recognized together with a corresponding increase in equity over the period in which the performance and/or service conditions are fulfilled. The cumulative expense for the Group's share-based compensation awards recognized at each reporting date until the vesting date reflects the extent to which the vesting period has expired and Management's best estimate of the number of instruments that will ultimately vest. The expense or credit in the Statement of Profit or Loss for a period represents the movement in cumulative expense recognized as at the beginning and end of that period.

In the event that equity instruments are granted conditionally upon an equal number of equity instruments granted in prior periods not being exercised, they are treated as a new grant for the current period and a modification of the equity instruments granted in the prior period.

When the terms of an equity-settled award are modified, the minimum expense recognized is the grant date fair value of the unmodified award, provided that the original terms of the award are met. An additional expense, measured as at the date of modification, is recognized for any modification that increases the total fair value of the share-based payment transaction, or is otherwise beneficial to the employee. Where an award is cancelled by the entity or by the counterparty, any remaining fair value of the award is expensed immediately in the Statement of Profit or Loss.

**Cash-settled awards**

The phantom share-based incentive programs established by the Group are settled in cash. The Restricted Share Units (RSU) awards to the board of directors may be settled in cash or in shares, at the choice of the participant, and are such treated as cash-settled awards. A liability is recognized for the fair value of cash-settled awards, measured initially and at each reporting date up to and including the settlement date, with changes recognized through

## 2.5 SHARE-BASED COMPENSATION COSTS (CONTINUED)

profit or loss at each reporting date. The fair value is expensed over the period until vesting date with recognition of a corresponding liability. The fair value is determined using the Monte-Carlo model, further details of which are presented below. The fair value of the cash-settled awards, which vest subject to obtaining a specified share price (i.e. market condition), is reported as compensation expense regardless of whether the share price condition is met if all other vesting conditions are met. For these awards, fair value is determined taking into account the probability of meeting the share price target. No expense is recognized for awards that do not ultimately vest. If the RSUs are finally exercised, the related liability is reclassified as equity.

**Estimate of inputs and assumptions used in share-based compensation valuation models**
Estimating the fair value of the Group's share-based compensation programs requires determination of the most appropriate valuation model, which depends on the terms and conditions of the respective award. This estimate also requires making assumptions to determine the most appropriate inputs to the valuation model, including the expected life of the award, expected volatility, dividend pay-out ratio, and risk-free interest rate. All references to share price relate to the Company's share price on Nasdaq Copenhagen.

**a) Long-term incentive program (equity-settled)**
In connection with the completion of the Company's initial public offering (IPO) on Nasdaq Copenhagen in November 2017, the Executive Management and Key Employees were offered to subscribe for Offer Shares ("Investment Shares") at the Offer Price for a maximum amount corresponding to approximately 15% (CMO) and 20% (CEO, CFO, and CSO) of their respective current annual base salaries.

Under the post-IPO long-term incentive program (2017 LTIP), the Executive Management as well as certain Key Employees of Orphazyme have subscribed to 14,875 ordinary shares (Investment Shares) at the offer price of DKK 80. In April 2018, a Key Employee subscribed to 4,300 Investment Shares at the then-current market price of DKK 67.5.

The participants in the 2017 LTIP may be allocated a number of shares in Orphazyme ("Performance Shares") at a price per Performance Share of DKK 1 at the end of a vesting period of four years from Orphazyme's first day of trading and official listing on Nasdaq Copenhagen. The number of Performance Shares shall be proportional to the potential increase in the price of Orphazyme's shares at the time of exercise compared to the offer price. The potential increase in the price of Orphazyme's shares will be calculated as the volume-weighted average share price as quoted on Nasdaq Copenhagen during the 10 trading days preceding the vesting date. The maximum allocation of Performance Shares will be six shares for the CEO and four shares for the other participants multiplied by the number of Investment Shares subscribed for in connection with the IPO. Performance Shares will be allocated on a linear scale with maximum allocation triggered by an 80% increase in share price, whereas no Performance Shares will be allocated if the price of Orphazyme's shares has increased 20% or less at the end of the vesting period. Among other things, vesting is also subject to the participants having maintained ownership of their Investment Shares and continued employment. Based on the number of Investment Shares subscribed for, a total maximum of 86,700 Performance Shares may be issued at the end of the vesting period.

In addition, subject to Board approval, the participants may also be allocated a number of shares in Orphazyme ("Matching Shares") at a price per Matching Share of DKK 1 in connection with the first anniversary of the subscription date of the Investment Shares. The number of Matching Shares shall be equal to the number of Investment Shares subscribed for and vesting will be subject to the participants having maintained ownership of their Investment Shares and continued employment during the one-year vesting period. By March 2019, all 19.175 Matching Shares under the 2017 LTIP vested in full and were issued against a nominal payment of DKK 1 per share.

In case of termination of a participant's employment, the participant may be designated a Good Leaver, which entitles him to receive Matching Shares and Performance Shares as if the participant was still employed with the Group.

In July 2019, the Company initiated a 2019 long-term investment program (2019 LTIP) for the Executive Management and certain Key Employees with the same terms and conditions as the 2017 LTIP, i.e. Matching Shares vesting over one year and Performance Shares vesting over four years. In July 2020, 31,250 matching shares fully vested and were issued against a nominal payment of DKK 1 per share. The maximum number of Performance Shares that can vest in July 2023 as part of the 2019 LTIP is 125.000.

In 2020, the Company initiated a 2020 long-term investment program (2020 LTIP) for the Executive Management and certain other employeeswith the same terms and conditions as the 2017 LTIP and the 2019 LTIP.  However, in case of termination of a

## 2.5 SHARE-BASED COMPENSATION COSTS (CONTINUED)

participant's employment and designation as a Good Leaver, the right to receive Matching Shares and Performance Shares will be prorated and calculated through the date of notice of termination. During 2020, awards were granted on four different grant dates shown in the table below. Matching Shares for all awards granted under the 2020 LTIP will fully vest in January 2021. The maximum number of Performance Shares that can vest in January 2024 as part of the 2020 LTIP is 489.757.

The fair value of all the LTIP awards was estimated using a Monte-Carlo simulation model at the respective grant dates, considering the terms and conditions on which the awards were granted.

The risk-free interest rate has been estimated based on Danish government bonds with similar maturities. As of November 2020, expected volatility has been determined based on the Company's own historical volatility, as the Company has been publicly traded for three years. Before November 2020, expected volatility was determined based on the historical volatility of comparable listed companies. The Company does not plan to pay out dividends in the foreseeable future.

The following table presents the fair value of each program and the inputs used in the valuation models at the respective grant dates:

| PROGRAM<br>GRANT DATE | 2020 LTIP<br>Dec 2020 | 2020 LTIP<br>Oct 2020 | 2020 LTIP<br>Sep 2020 | 2020 LTIP<br>Aug 2020 |
|---|---|---|---|---|
| Fair value at the measurement date (DKK 000) | 446 | 4,464 | 1,482 | 44,126 |
| Dividen yield (%) | - | - | - | - |
| Expected volatility (%) | 45.9% | 56.5% | 56.1% | 55.4% |
| Risk-free interest rate (%) | (0.66%) | (0.60%) | (0.59%) | (0.54%) |
| Expected life of awards (years) | 3.06 | 3.24 | 3.24 | 3.35 |
| Weighted average share price (DKK) | 56.30 | 68.50 | 83.50 | 90.10 |

| PROGRAM<br>GRANT DATE | 2019 LTIP<br>Aug 2019 | 2017 LTIP<br>Apr 2018 |
|---|---|---|
| Fair value at the measurement date (DKK 000) | 6,214 | 714 |
| Dividen yield (%) | - | - |
| Expected volatility (%) | 51.8% | 41.8% |
| Risk-free interest rate (%) | (0.70%) | (0.28%) |
| Expected life of awards (years) | 3.42 | 3.58 |
| Weighted average share price (DKK) | 62.6 | 67.5 |

The following table presents the weighted average remaining contractual life in years of the LTIP awards outstanding at December 31:

| PROGRAM | 2020 | 2019 |
|---|---|---|
| 2020 LTIP | 3.0 | - |
| 2019 LTIP | 2.7 | 3.7 |
| 2017 LTIP | 0.9 | 1.9 |

## 2.5 SHARE-BASED COMPENSATION COSTS (CONTINUED)

The exercise price for each LTIP award outstanding as of December 31, 2020 was DKK 1 (2019: DKK 1).

| (DKK 000) | Executive Management | Key Employees | Total awards | Awards exercisable |
|---|---|---|---|---|
| **Outstanding at December 31, 2017** | **9,000** | **5,875** | **14,875** | **-** |
| Granted | - | 4,300 | 4,300 | - |
| Exercised | | | | - |
| Expired | | | | - |
| Forfeited | | | - | - |
| **Outstanding at December 31, 2018** | **9,000** | **10,175** | **19,175** | **14,875** |
| Granted | 6,250 | 25,000 | 31,250 | - |
| Exercised | (9,000) | (10,175) | (19,175) | - |
| Expired | - | - | - | - |
| Forfeited | - | - | - | - |
| **Outstanding at December 31, 2019** | **6,250** | **25,000** | **31,250** | **-** |
| Granted | 52,865 | 119,623 | 172,488 | - |
| Exercised | (6,250) | (25,000) | (31,250) | - |
| Expired | - | - | - | - |
| Forfeited | - | (937) | - | - |
| **Outstanding at December 31, 2020** | **52,865** | **118,686** | **172,488** | **172,488** |

The table below summarizes the activity related to the LTIP awards for the years ended December 31:

For the year ended December 31, 2020, DKK 17.9 million (2019: DKK 2.1 million; 2018: DKK 2.1 million) was recognized as compensation expense related to the LTIP awards, with DKK 6.3 million recognized as research and development expenses and DKK 11.6 million recognized as general and administrative expenses. Of the total expense, DKK 8.2 million (2019: DKK 0.7 million; 2018: DKK 1.1 million) is attributed to the Executive Management.

**b) Phantom share-based incentive program (cash-settled)**

In June 2018, Orphazyme introduced a four-year phantom share-based incentive program (the "2018 Phantom Shares Program") for all employees other than the Executive Management and Key Employees under the LTIP.

In August 2019, Orphazyme initiated a 2019 Phantom Shares Program with the same terms and conditions as the 2018 Phantom Shares Program. In December 2020, Orphazyme initiated a 2020 Phantom Shares Program with the same terms and conditions as the 2018 and 2019 Phantom Shares Programs.

The Phantom Shares Programs are based on the share price of the Company and entitles the participants to a potential cash bonus if there has been an increase of at least 20% in Orphazyme's share price compared to the entry price at the grant date. The Phantom Shares Programs will not have any dilutive effect on the shareholders of Orphazyme as the phantom shares do not constitute or qualify for actual shares in Orphazyme.

The overall objectives of the Phantom Shares Programs are (i) to retain qualified employees, (ii) to create long-term incentive for the participants, and (iii) to align the interests of the employees with those of Orphazyme's shareholders. Each employee participating in the program earns the right to a certain number of phantom shares per month, depending on the employee's position. Subject to any adjustments to the Phantom Shares Programs made by the Board of Directors due to, for example, changes in Orphazyme's share capital structure or other significant events, each employee will be eligible to receive up to a total of 144 or 288 phantom shares under the program. By the end of each calendar year of the four-year program, the participants will have earned phantom shares free of charge.

The entry price per phantom share for the 2018 and 2019 Phantom Programs was DKK 61 and for the 2020 Phantom Program was DKK 71.2. The entry price has been calculated on the basis of the volume-weighted average closing price of Orphazyme's share on Nasdaq Copenhagen during a period of 10 trading days prior to the introduction of the respective Phantom Shares Program. The phantom shares will automatically be settled in cash at the end of January 2023 for the 2018 Phantom Shares Program, at the end of January 2024 for the 2019 Phantom Shares Program and at the end of January 2024 for the 2020 Phantom Shares Program by subtracting the entry price per share from the market price per share and multiplying the change by the total number of granted phantom shares, but only if Orphazyme's market price per share at that date exceeds the entry price per share by at least 20%. The market price per share will be based on the volume-weighted average closing price of Orphazyme's shares on Nasdaq Copenhagen during a period of 10 trading days prior to the settlement of the phantom shares.

The employee's cash award for each program is capped and cannot exceed a gross amount of DKK 37,500 or DKK 75,000 per employee per program, depending on the

## 2.5 SHARE-BASED COMPENSATION COSTS (CONTINUED)

number of phantom shares allocated to the respective employee under the program. Based on the number of participants in the Phantom Shares Programs as of December 31, 2020 and 2019, the programs are expected to consist of up to a total of 41,351 and 12,750 phantom shares, respectively.

As of December 31, 2020, all phantom shares granted under the Phantom Shares Program were only granted to employees of Orphazyme. No phantom shares were forfeited or expired, and none of the phantom shares were eligible for exercise.

As the Phantom Shares Programs are cash-settled, the fair value of the phantom shares granted as part of the program is estimated at each reporting date. For the year ended December 31, 2020, an aggregate amount of DKK 0.1 million (2019: DKK 0.3 million; 2018: DKK 39 thousand) was recognized as compensation expense related to the Phantom Shares Programs, with a corresponding amount recognized as a non-current liability (Note 3.6).

The risk-free interest rate has been estimated based on Danish government bonds with similar maturities. As of November 2020, expected volatility has been determined based on the Company's own historic volatility, as the Company has been publicly traded for three years. Before November 2020, expected volatility was determined based on the historical volatility of comparable listed companies.

The following table presents the inputs to the Monte-Carlo model used to estimate the fair values of the phantom shares as of year-end, when the cash-settled programs are re-valued:

| VALUATION DATE: | December 31, 2020 | | | December 31, 2019 | |
|---|---|---|---|---|---|
| Program | 2020 Program | 2019 Program | 2018 Program | 2019 Program | 2018 Program |
| Fair value at valuation date (DKK 000) | 406 | 293 | 160 | 347 | 205 |
| Dividend yield (%) | - | - | - | - | - |
| Expected volatility (%) | 47.1% | 47.3% | 54.3% | 57.4% | 57% |
| Risk-free interest rate (%) | (0.59%) | (0.61%) | (0.61%) | (0.50%) | (0.63%) |
| Expected life of awards (years) | 4.08 | 3.08 | 2.08 | 4.00 | 3.08 |
| Weighted average share price (DKK) | 67.10 | 67.10 | 67.10 | 72.40 | 68.60 |

**c) Restricted Share Units (cash-settled)**
According to the terms and conditions of the Restricted Share Units program (RSU), directors may annually be granted a number of RSUs with a value corresponding to up to 50% of the participant's fixed annual base fee as member of the Board of Directors, not including committee membership fees. The value is calculated on the basis of the volume-weighted average share price of Orphazyme's shares as quoted on Nasdaq Copenhagen during the ten trading days preceding the grant date. The RSUs vest from the grant date to the date of the next annual general meeting. Upon vesting, RSUs may be exercised within a period of twelve months from vesting (Exercise Period) at a price corresponding to the volume-weighted average share price during the ten trading days preceding the grant date (Exercise Price). In the event of a participant's resignation from the Board of Directors, any unvested RSUs will lapse without any rights of compensation. A decision not to be re-elected is not a resignation from the Board of Directors.

The RSUs are classified as a cash-settled program, as the Board of Directors may choose to settle any vested RSUs in cash. In such event, the cash settlement amount is based on the difference between the Exercise Price and the volume-weighted average share price as quoted on Nasdaq Copenhagen during the ten trading days preceding the first day of the Exercise Period.

In August 2019, Restricted Share Units (2019 RSUs) were granted to members of the Board of Directors. These RSUs fully vested in March 2020. During 2020 certain board members exercised their RSUs. The remaining RSUs expire in March 2021 if not exercised or paid out in cash. Also in March 2020, the 2020 RSU program was announced, granting the Board of Directors an aggregate of 15,177 RSUs under similar terms and conditions as the 2019 RSUs.

In September 2020, a new RSU incentive program was announced (2020-2 RSU program), which comprised 22,993 RSUs in total, including an on-boarding grant to a new board member in accordance with the Company's remuneration policy. The 2020-2 RSU program runs in parallel with the 2020 RSU program and board members can only exercise RSUs under one of the programs. In December 2020, 4,351 RSUs (2020-3 RSU program) were granted to the Chairman of the Board as part of a consultancy agreement (see Note 4.6).

The fair value of all RSUs was calculated using a Black-Scholes valuation model with the inputs shown in the following table. As the RSUs may be settled in cash, we have re-valued them as of year-end with

## 2.5 SHARE-BASED COMPENSATION COSTS (CONTINUED)

updated inputs and recognized a cumulative share-based compensation expense in the amount of DKK 0.8 million (2019: 0.1 million; 2018: 0) and a corresponding short-term liability as of December 31, 2020. The Exercise Period for all 2020 RSUs is one year and for valuation purposes we have assumed exercise three months upon full vesting.

As of December 31, 2020 no RSUs were forfeited or expired, and 5,781 RSUs were eligible for exercise.

**d) Sign-on bonus shares to CEO**

As part of the CEO service agreement, Kim Stratton was granted 58,000 ordinary shares, which would vest if the Company's share price increased to DKK 125 per share within three years from the date of employment. The total award consisted of (i) 6,000 shares provided that our share price increased to DKK 75 per share, (ii) 12,000 shares provided that our share price increased to DKK 100 per share, and (iii) 40,000 shares provided that our share price increased to DKK 125 per share. The target prices were achieved and the 58,000 ordinary shares were issued to Ms. Stratton subsequent to December 31, 2020 (see Note 4.8).

The shares were valued at grant date, October 2019, using a Monte Carlo model due to the market conditions for vesting. The risk-free interest rate used in the model has been estimated based on Danish government bonds with similar maturities; expected volatility has been determined based on the historic volatility of comparable listed companies; the expected life of the award was 3 years, equal to the term of the award; the estimated dividend yield was zero; and the weighted average share price was DKK 55.60. The total valuation of the award at grant date was DKK 1.9 million. The total share-based compensation expense was classified as administrative and it was recognized in full during 2020, as the target prices were achieved in 2020.

**e) Bonus shares issued to KLSDC and UCL in connection with the license agreement**

Please see Note 3.1.

The following table presents the inputs to the Black-Scholes model used to estimate the fair value of the 2020 RSUs at December 31:

| | December 31, 2020 | 2019 |
|---|---|---|
| **Program** | **2020 RSUs** | **2019 RSUs** |
| Fair value at valuation date (DKK 000) | 1,913 | 232 |
| Dividend yield (%) | - | - |
| Expected volatility (%) | 45.9% | 45.9% |
| Risk-free interest rate (%) | (0.57%) | (0.73%) |
| Expected life of awards (years) | 0.50 | 0.25 |
| Weighted average share price (DKK) | 67.10 | 72.4 |

The following amounts were recognized as share-based compensation for the years ended December 31:

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| Share-based compensation included in R&D | 7,260 | 635 | 719 |
| Share-based compensation included in G&A | 20,845 | 1,914 | 1,426 |
| **Total share-based compensation expense recognized** | **28,105** | **2,549** | **2,145** |

## 2.6 FINANCIAL INCOME AND FINANCIAL EXPENSES

### § ACCOUNTING POLICIES

Financial income and expenses include interest income and expense, gains and losses due to changes in foreign exchange rates and other immaterial miscellaneous items.

Beginning January 1, 2019, interest expense related to the right-of-use assets and interest expense related to the Loan Agreement are also recognized as financial expenses.

The following table presents the various items of financial income and expense recognized for the years end December 31:

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| Interest income on cash balances | 45 | 316 | 5 |
| Foreign currency exchange gains | 1,649 | - | - |
| Gain on embedded call option (Note 3.6) | 750 | - | - |
| **Total financial income** | **2,444** | **316** | **5** |
| Interest expense on Loan Agreement (Note 3.6) | 9,921 | 3,239 | - |
| Write-off of transaction costs for Loan Agreement tranche 2 (Note 3.6) | - | 1,678 | - |
| Loss on embedded call option (Note 3.6) | - | 354 | - |
| Interest expense on lease liabilities (Note 3.2) | 567 | 351 | - |
| Loss on lease modification (Note 3.2) | - | 216 | - |
| Interest expense on cash balances | 3,626 | 1,213 | 2,824 |
| Foreign currency exchange loss | 14,805 | 229 | 490 |
| Bank fees and other charges | 152 | 79 | 139 |
| **Total financial expenses** | **29,071** | **7,359** | **3,453** |

## 2.7 INCOME TAXES

### § ACCOUNTING POLICIES

Income tax benefit includes the current benefit due from the current period's taxable loss and deferred tax adjustments. The benefit is comprised primarily of refundable tax credits for costs incurred in connection with research and development activities under the Danish Tax Credit Regime. Income tax expense relates to tax imposed on income recognized in our subsidiaries in the United States and Switzerland as a result of our transfer pricing agreements. Corporation tax receivable is recognized in the balance sheet as the tax benefit computed on the taxable loss for the year, adjusted for any changes to the prior year benefit due to changes in the taxable loss of prior years and for any taxes already paid or refunded.

Deferred tax is measured using the balance sheet liability method on all temporary differences between the carrying amount and the tax value of assets and liabilities, with the exception of temporary differences occurring at the time of acquisition and liabilities neither affecting the result of operation nor the taxable income.

As of December 31, 2020 and 2019, there were no tax audits in process nor has management been notified of any pending tax audit.

## 2.7 INCOME TAXES (CONTINUED)

**Judgement regarding the recognition of the deferred tax assets related to taxable losses to be carried forward**

Orphazyme is subject to income taxes in Denmark, Switzerland and the U.S.A. The Company recognizes deferred income tax assets if it is probable that sufficient taxable income will be available in the future against which the temporary differences and unused tax losses can be utilized. Significant judgment is required to determine the amount of deferred tax assets that may be recognized, based upon the likely timing and the level of future taxable profits together with future tax planning strategies. This judgment is made periodically after considering current facts and circumstances, budgets and business plans as well as the risks and uncertainty associated with the Company's ability to successfully commercialize and defend its intellectual property. After consideration of these factors, Management has concluded that as regulatory approval has not yet been obtained as of December 31, 2020, the deferred income tax assets related to taxable losses carried forward in Denmark do not meet the criteria for being recognized as assets in the Statement of Financial Position. However, Management has concluded that the deferred income tax asset related to income in the United States is recoverable, as the US entity will continue to be profitable and therefore will utilize the losses carried forward.

The Company's tax losses can be carried forward infinitely subject to the general rules on limited deductibility due to ownership changes. In Denmark, the Company's ability to use tax loss carry forwards in any one year is limited to 100% of the first DKK 8.4 million of taxable income plus 60% of taxable income above DKK 8.4 million.

For the years ended December 31, 2020, 2019, and 2018, the Company has unrecognized net tax loss carry-forwards in the Danish entity in the amount of DKK 877 million, DKK 425 million, and DKK 280 million respectively.

Management periodically evaluates positions taken in tax returns with respect to situations in which applicable tax regulations are subject to interpretation or uncertainty and establishes provisions, where appropriate. To date, there have not been any provisions established for uncertain tax positions.

The following table presents the total income tax benefit for the years ended December 31:

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| Current tax benefit on net loss | 136,845 | 75,459 | 51,722 |
| Adjustment prior years | (1,065) | - | - |
| Tax credit research and development expenses | 5,500 | 5,500 | 5,500 |
| Change in unrecognized deferred tax before tax credit | (142,115) | (74,961) | (51,850) |
| Permanent differences | 2,750 | (498) | 128 |
| **Total income tax benefit for the year** | **1,915** | **5,500** | **5,500** |

The following table presents the reconciliation of the effective tax rate to the statutory corporate income tax rate in Denmark.

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| Net loss before tax | (635,161) | (342,997) | (235,100) |
| Corporate income tax rate in Denmark | 22% | 22% | 22% |
| **Computed income tax benefit** | **139,735** | **75,459** | **51,722** |
| *Tax effect of:* | | | |
| *Adjustments prior years* | (1,065) | - | |
| Other non-deductible expenses, including U.S. listing-related costs and share-based compensation | 2,750 | (498) | 128 |
| Effect of different tax rate | (673) | - | - |
| Deferred tax asset not recognized after tax credit | (138,832) | (69,461) | (46,350) |
| **Total income tax benefit for the period** | **1,915** | **5,500** | **5,500** |

The following table presents the carrying amount of deferred tax in the Statement of Financial Position:

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| Tax deductible losses | 192,837 | 93,484 | 61,647 |
| Deferred tax on intangible assets | 112,192 | 74,050 | 35,887 |
| Other temporary differences | 8,174 | 758 | 738 |
| | 313,203 | 168,292 | 98,272 |
| Deferred tax asset not recognized | 311,138 | 168,292 | 98,272 |
| **Carrying amount included in the Statement of Financial Position** | **2,065** | **-** | **-** |

SECTION 3

# ASSETS AND LIABILITIES

**Section 3 presents details of the assets and liabilities that form the basis of Orphazyme's activities, including Licenses, Right-of-Use Assets and Lease Liabilities, Property, Plant, and Equipment, Prepayments, Deposits, and Other Receivables, Financial Assets and Liabilities, Cash, and Contractual Obligations and Contingencies.**

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## 3.1 INTANGIBLE ASSETS

### § ACCOUNTING POLICIES

Intangible assets comprise software development costs and license rights to develop and commercialize products and are acquired separately and measured on initial recognition at cost. Software assets consist of implementation costs to get ERP cloud computing arrangements ready for use, as long as they meet the requirements of IAS 38, Intangible Assets. These cloud computing arrangements begin to be amortized when they are ready for intended use and are amortized over seven years.

For acquisition of intangible rights involving equity-settled share-based payment transactions, Management measures the fair value of the rights received and the corresponding increase in equity by reference to the fair value of the rights received, unless that fair value cannot be estimated reliably. If Management cannot estimate reliably the fair value of the rights received, it measures the fair value and the corresponding increase in equity by reference to the fair value of the equity instruments granted.

Following initial recognition, intangible assets are carried at cost less any accumulated amortization and accumulated impairment losses. The useful lives of intangible assets are assessed as either finite or indefinite. Intangible assets with finite lives such as software and license rights to develop and commercialize products are amortized over the useful economic life and assessed for impairment whenever there is an indication that the intangible asset may be impaired.
The amortization period and the amortization method for an intangible asset with a finite useful life are reviewed at least at the end of each reporting period. Changes in the expected useful life or the expected pattern

**Estimate of the fair value of licenses**
Licenses contains an agreement entered into with the University of Kansas and University College London, in which the Company will obtain access to data and knowhow generated in the course of research in connection with the IBM trial. Consideration for the license is to be paid out by issuing new shares to the contract partners for a value corresponding to the costs incurred during the preceding calendar year. The valuation of the license upon the execution of the agreement involves uncertainty and was estimated by Management based on the expected costs over the contract period. In addition, the estimation of the duration of a license agreement at times involves uncertainty if termination is dependent on a time limit after successful commercialization. Management has considered potential commercialization dates and will re-assess this estimate on an ongoing basis.

of consumption of future economic benefits embodied in the asset are considered to modify the amortization period or method, as appropriate, and are treated as changes in accounting estimates. The amortization expense on intangible assets with finite lives is recognized in the Statement of Profit or Loss in the expense category that is consistent with the function of the intangible assets.

**CytRx Asset Purchase Agreement**

In May 2011, Orphazyme entered into an Asset Purchase Agreement with the US biopharmaceutical company CytRx. Pursuant to this agreement, CytRx sold and transferred certain preclinical and clinical data, patents and other intellectual property rights, and other assets, including contractual rights and obligations relating to a portfolio of chemical compounds, including arimoclomol, to Orphazyme. Under the terms of the Asset Purchase Agreement, Orphazyme agreed to make future payments to CytRx that were contingent upon the achievement of specified clinical, regulatory, and sales milestones. These payments are further disclosed in Note 3.8.

In 2016, the Company paid CytRx USD 0.1 million (DKK 0.6 million) for achievement of a clinical milestone for the first product candidate. In August 2018, the Company made a milestone payment of USD 250,000 (DKK 1.6 million) upon the enrollment of the first patient in the ALS clinical trial. The Company capitalizes amounts paid to CytRx as an acquired license right if the recognition criteria under IAS 38 is met. Management assesses that the consideration paid reflects market expectations about the probability that future economic benefits will flow to the Company. The acquired license is not being amortized until approval of the underlying asset has been received from regulatory authorities.

The Asset Purchase Agreement further includes sales milestones and royalty payments to be made by Orphazyme based on a specified percentage of any eventual net sales of products containing one of the compounds purchased. In addition, under the terms of the Asset Purchase Agreement, the Company was assigned and became party to a royalty agreement with ALS Charitable Remainder Trust pursuant to which the Company is obliged to pay a 1% royalty to the ALS Charitable Remainder Trust on global net sales of products to treat ALS. Orphazyme has no liabilities prior to the occurrence of future sales of products and accordingly neither such liabilities nor contingent consideration have been recognized as part of the license agreement.

**License Agreement with KLSDC and UCL**

In 2017, the Company entered into a license agreement with KU Center for Technology Commercialization Inc., University of Kansas, Kansas Life Sciences Development Company, Inc., ("KLSDC") and UCL Business PLC ("UCL") granting Orphazyme the right to develop and commercialize products under all data generated in the course of the on-going Phase 2/3 clinical trial on arimoclomol for the treatment of IBM worldwide. The total consideration for the license is to be paid out in bonus shares to KLSDC and UCL up to an aggregate value of USD 2.5 million (DKK 15.8 million), depending on the amount of grants awarded to KLSDC and UCL for use in the trial. At the time the license agreement was executed, Management estimated the aggregate amount of the funding to be received by KLSDC and UCL to be USD 1.6 million (DKK 10 million), which has been recognized as an intangible asset (License) with a corresponding increase in equity reserves (Share-based compensation - acquisition of intangible assets).

Consideration to KLSDC and UCL is payable in shares of the Company ("Bonus Shares") each January and is based on incurred costs reported by KLSDC and UCL for the previous year. In January 2020, 20,650 (2019: 26,060) Bonus Shares were issued to KLSDC and UCL based on aggregate costs incurred by KLSDC and UCL in the amount of USD 0.3 million (DKK 2.2 million) (2018: USD 0.2 million (DKK 1,197 million)). The Bonus Shares were derived based on the average 30-day closing price of Orphazyme's shares at the date of issuance. At the time of the share issuance the equity reserve was decreased by DKK 2.1 million, which represents the market value of the shares issued. See Note 4.8 for Bonus Shares to be issued in 2021 related to the incurred costs reported by KLSDC and UCL for the year 2020.

Under the terms of the license agreement, Orphazyme shall furthermore pay an aggregate royalty of a low single-digit percentage of net sales of products sold for the treatment of IBM. Orphazyme expects to generate income from such products sold for the treatment of IBM which will exceed any royalty payments due. Orphazyme has no liabilities prior to the occurrence of future sales of products sold for the treatment of IBM and accordingly, neither such liabilities nor contingent considerations have been recognized as part of the rights acquired.

The license is being amortized over the duration of the license agreement, which has been estimated to be approximately 14 years. The remaining life of the license is 10.8 years. Amortization expense for the years ended December 31, 2020 and 2019 amounts to DKK 0.7 million and DKK 0.7 million, respectively, and is recognized within research and development expenses.

**License Agreement with the University of Miami**

In September 2019, the Company entered into an exclusive license agreement with the University of Miami. Pursuant to the exclusive license agreement, the Company was granted a global royalty-bearing, exclusive license to all data, know-how, inventions and technology generated by the University of Miami and certain other institutions in a Phase 2 clinical trial of arimoclomol in ALS with the A4V SOD1 mutation to research, develop, make, use or sell certain pharmaceutical products or processes containing arimoclomol.

## 3.1 INTANGIBLE ASSETS (CONTINUED)

Under the terms of the exclusive license agreement, the Company made an up-front cash payment of $75,000 (DKK 0.5 million) and further agreed to make certain future payments, including (i) a development milestone payment of $1,150,000 (DKK 7.7 million) upon receiving regulatory approval for a pharmaceutical product containing arimoclomol for which the intended indication is ALS if the institution's Phase 2 clinical trial results were used in support of such regulatory approval, (ii) annual license fees from 2023 until the earlier of 2033 or termination of the agreement for a maximum aggregate amount of $570,000 (DKK 3.8 million), and, (iii) beginning on the date of first commercial sale by the Company, its affiliates or sublicensees of a licensed product or licensed process in a country, a low single-digit royalty on net sales of licensed products or licensed processes on a product-by-product and country-by-country basis for a period of ten years thereafter unless the agreement is terminated earlier. Any annual license fees will be creditable against other payments due in the same calendar year.

Orphazyme has no liabilities prior to the occurrence of future sales of products and accordingly neither such liabilities nor contingent consideration have been recognized as part of the license agreement.

The up-front cash payment was capitalized as an acquired license right, which is not being amortized until approval of the underlying asset has been received from regulatory authorities.

The following table presents the cost and respective amortization of software and licenses held by Orphazyme:

| DKK 000 | Software | Licenses | Total |
|---|---|---|---|
| Cost at December 31, 2018 | - | 11,575 | 11,575 |
| Additions | - | 508 | 508 |
| Cost at December 31, 2019 | - | 12,083 | 12,083 |
| Additions | 2,736 | - | 2,736 |
| **Cost at December 31, 2020** | **2,736** | **12,083** | **14,819** |
| Accumulated amortization at December 31, 2018 | - | 831 | 831 |
| Amortization expense | - | 713 | 713 |
| Accumulated amortization at December 31, 2019 | - | 1,544 | 1,544 |
| Amortization expense | 109 | 712 | 821 |
| **Accumulated amortization at December 31, 2020** | **109** | **2,256** | **2,365** |
| **Net carrying value at** | | | |
| December 31, 2019 | - | 10,539 | 10,539 |
| December 31, 2020 | 2,627 | 9,827 | 12,454 |

## 3.2 LEASES

### § ACCOUNTING POLICIES

On January 1, 2019, Orphazyme adopted IFRS 16, Leases, using the modified retrospective method. At contract inception, the Group assesses whether a contract is, or contains, a lease. A contract is, or contains, a lease if the contract conveys the right to control the use of an identified asset for a period of time in exchange for consideration. The Group is party to lease agreements only in which it is a lessee and not a lessor.

As a lessee, the Group applies a single recognition and measurement approach for all leases, except for short-term leases and leases of low-value assets. At inception or on reassessment of a contract that contains a lease component, the Group allocates the consideration in the contract to each lease component on the basis of their relative stand-alone prices.

The Group recognizes lease liabilities to make lease payments and right-of-use assets representing the right to use the underlying assets.

**Right-of-use assets**

The Group recognizes right-of-use assets at the commencement date of the lease (i.e., the date the underlying asset is available for use). Right-of-use assets are measured at cost, less any accumulated depreciation and impairment losses, and adjusted for any remeasurement of lease liabilities. The cost of right-of-use assets includes the amount of lease liabilities recognized, initial direct costs incurred, and lease payments made at or before the commencement date less any lease incentives received.

Right-of-use assets are depreciated on a straight-line basis over the shorter of the lease term and the estimated useful life of the underlying asset. If ownership of the leased asset transfers to the Group at the end of the lease term or the cost reflects the exercise of a purchase option, depreciation is calculated using the estimated useful life of the asset, which for the operating equipment under lease is ten years. The right-of-use assets are also subject to impairment.

**Lease liabilities**

At the commencement date of the lease, the Group recognizes lease liabilities measured at the present value of lease payments to be made over the lease term. The lease payments include fixed payments less any lease incentives receivable, variable lease payments that depend on an index or a rate, and amounts expected to be paid under residual value guarantees. The lease payments also include the exercise price of a purchase option reasonably certain to be exercised by the Group and payments of penalties for terminating the lease, if the lease term reflects the Group exercising the option to terminate. Variable lease payments that do not depend on an index or a rate are recognized as expenses in the period in which the event or condition that triggers the payment occurs. In calculating

## 3.2 LEASES (CONTINUED)

the present value of lease payments, the Group uses its incremental borrowing rate at the lease commencement date because the interest rate implicit in the lease is not readily determinable. After the commencement date, the amount of lease liabilities is increased to reflect the accretion of interest and reduced for the lease payments made. In addition, the carrying amount of lease liabilities is remeasured if there is a modification, a change in the lease term, a change in the lease payments (e.g., changes to future payments resulting from a change in an index or rate used to determine such lease payments) or a change in the assessment of an option to purchase the underlying asset. The Group's non-current lease liabilities are included as a separate line item on the Group's consolidated balance sheet and the current portion of lease liabilities is included in Other current liabilities.

**Short-term leases and leases of low-value assets**

The Group applies the short-term lease recognition exemption to its short-term leases of machinery and equipment (i.e., those leases that have a lease term of 12 months or less from the commencement date and do not contain a purchase option). It also applies the lease of low-value assets recognition exemption to leases of office equipment that are considered to be low value. Lease payments on short-term leases and leases of low-value assets are recognized as expense on a straight-line basis over the lease term.

**Lease modifications**

Lease modifications are accounted for at the effective date of modification, which is the date when both parties agree to the lease modification. Modifications are accounted for either as a separate lease or as a remeasurement of the initial lease. A modification is accounted for as a separate lease if both of the following conditions are met: (a) the modification increases the scope of the lease by adding the right to use one or more underlying assets; and (b) the consideration for the lease increases by an amount equivalent to the stand-alone price for the underlying asset. For a modification that is not a separate lease, the lease liability is remeasured using a discount rate determined at the effective date of the modification.

The Group has lease contracts for its headquarters in Copenhagen and for machinery used in its operations. The lease terms range from three to five years. During 2019, the lease contract for its headquarters in Copenhagen was modified to include additional space, a true-up to market value for the lease as a whole, and an extension of the whole lease term. The modification was accounted for as a change in the scope of the existing lease and therefore the initial lease was remeasured on the effective date of the modification at the weighted average incremental borrowing rate of 5.38%. The effect on the right-of-use assets, lease liabilities and the Statement of Profit or Loss is disclosed in the tables below. During 2020, the lease contract for its headquarters in Copenhagen was modified to include additional space, which was accounted for as a separate lease addition. Furthermore, in June 2020 the Company entered a new lease contract for its US office premises that expires September 2025.

**Estimate relating to the incremental borrowing rate**

The Group cannot readily determine the interest rate implicit in its leases, therefore it uses its incremental borrowing rate to measure lease liabilities. This is the rate of interest that the Group would have to pay to borrow over a similar term, and with a similar security, the funds necessary to obtain an asset of a similar value to the right-of-use asset in a similar economic environment. As there are no observable rates available for such a rate, the Group estimates its incremental borrowing rate using observable inputs, such as market interest rates, and is required to make certain entity-specific estimates.

## 3.2 LEASES (CONTINUED)

The following table presents the carrying amounts of right-of-use assets recognized and the movements during the period:

| (DKK 000) | Office buildings | Operating equipment | Total |
|---|---|---|---|
| At January 1, 2019 | 13,006 | - | 13,006 |
| Additions | - | 4,008 | 4,008 |
| Depreciation expense | (1,858) | (200) | (2,058) |
| Modifications | (1,053) | - | (1,053) |
| **At December 31, 2019** | **10,095** | **3,808** | **13,903** |
| Additions | **3,963** | **-** | **3,963** |
| Depreciation expense | **(2,606)** | **(401)** | **(3,007)** |
| Exchange rate adjustments | **-** | **-** | **-** |
| **At December 31, 2020** | **11,452** | **3,407** | **14,859** |

The following table presents the carrying amounts of lease liabilities and the movements during the period:

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| At January 1, 2019 | 12,689 | 13,006 |
| Additions | 3,963 | 4,008 |
| Accretion of interest | 567 | 351 |
| Payments | (3,678) | (3,838) |
| Exchange rate adjustments | (7) | - |
| Modifications | - | (838) |
| **At December 31, 2019** | **13,534** | **12,689** |
| Current | 3,657 | 2,876 |
| Non-current | 9,877 | 9,813 |

The maturity analysis of lease liabilities is disclosed in Note 3.6.

The following are the amounts recognized in the Statement of Profit or Loss:

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| Depreciation expense of right-of-use assets (R&D) | 2,441 | 1,847 | - |
| Depreciation expense of right-of-use assets (G&A) | 566 | 211 | - |
| Interest expense on lease liabilities | 567 | 351 | - |
| Loss on lease modification | - | 216 | - |
| **Total amount recognized in the Statement of Profit or Loss** | **3,574** | **2,625** | **-** |

## 3.3 PROPERTY, PLANT, AND EQUIPMENT

### § ACCOUNTING POLICIES

Property, plant, and equipment includes IT, lab and other equipment, furniture and leasehold improvements that are measured at cost less accumulated depreciation and impairment losses. Cost includes the acquisition price and costs directly related to the acquisition until the time the asset is ready for use. The residual value of equipment is not material. Depreciation is calculated on a straight-line basis over the expected useful life of the asset, being 3-5 years for equipment and furniture. Leasehold improvements are depreciated over the shorter of the useful life of the improvement or the remaining lease term. The useful life of assets and method of depreciation are reviewed by management at least each year-end or more often based on changes in facts and circumstances. Changes in useful lives or residual values are adjusted prospectively as changes in accounting estimates. In addition, the Company has fully depreciated equipment still in use.

Property, plant, and equipment is required to be tested for impairment when there are impairment indicators present. Impairment tests are conducted at the individual asset level, or at the lowest level for which separately identifiable cash flows for groups of assets exist. Impaired assets or asset groups are written down to their recoverable amount, which is the higher of the value in use and the net realizable value of the asset or asset group, with impairment charges allocated proportionately to the assets within the impaired asset group.

The following table presents the Company's Property, plant and equipment as of the years presented:

| (DKK 000) | Furniture and equipment | Leasehold improvements | Total |
|---|---|---|---|
| Cost at December 31, 2018 | 4,419 | 402 | 4,821 |
| Additions | 1,113 | 1,664 | 2,777 |
| Disposals | - | - | - |
| Cost at December 31, 2019 | 5,532 | 2,066 | 7,598 |
| Additions | 1,840 | 525 | 2,365 |
| **Cost at December 31, 2020** | **7,372** | **2,591** | **9,963** |
| Accumulated depreciation at December 31, 2018 | 2,761 | 120 | 2,881 |
| Depreciation expense | 852 | 180 | 1,032 |
| Accumulated depreciation at December 31, 2019 | 3,613 | 300 | 3,913 |
| Depreciation expense | 1,004 | 367 | 1,371 |
| Exchange rate adjustments | (8) | - | (8) |
| **Accumulated depreciation at December 31, 2020** | **4,609** | **667** | **5,276** |
| **Net carrying value at** | | | |
| December 31, 2019 | 1,919 | 1,766 | 3,685 |
| December 31, 2020 | **2,763** | **1,924** | **4,687** |

There has been no impairment of property, plant and equipment for the years ended December 31, 2020 and 2019. Depreciation expense is included within operating loss as follows:

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| Research and development expenses | 887 | 544 | 580 |
| General and administrative expenses | 484 | 489 | 74 |
| **Total depreciation expense** | **1,371** | **1,033** | **654** |

# 3.4 PREPAYMENTS, DEPOSITS, AND OTHER RECEIVABLES

## § ACCOUNTING POLICIES

**Prepayments**
Prepayments include advance payments made to vendors that will be incurred and expensed in subsequent financial reporting periods. When the period for full expense recognition is longer than one year from the balance sheet date, the portion to be expensed subsequent to one year is classified as non-current.

**Deposits**
Deposits include advance payments made to vendors to be settled upon completion of the underlying contract. When the contract term is longer than one year from the balance sheet date, the deposit is classified as non-current.

**Other receivables**
Other receivables include current and non-current amounts due to the Company.

**Sales tax**
Expenses and assets are recognized net of the amount of sales tax, except:

- when the sales tax incurred on a purchase of assets or services is not recoverable from the taxation authority, in which case, the sales tax is recognized as part of the cost of acquisition of the asset or as part of the expense item, as applicable
- when receivables and payables are stated with the amount of sales tax included.

**Estimate of prepayments related to clinical trial development costs**
As explained in Note 2.1, Orphazyme incurs substantial costs associated with clinical trials related to its development programs and there is a high degree of estimation involved in accounting for clinical trial development costs. In particular, certain CROs and vendors are paid upfront in connection with clinical activities and Management is required to estimate the timing of the prepayment release to expense. This expense for the year is estimated by using an expense model, as described in Note 2.1.

The net amount of sales tax recoverable from, or payable to, the taxation authority is included as part of receivables or payables in the Statement of Financial Position.

The following items comprised non-current prepayments and deposits as of December 31:

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Deposits with vendors | 500 | 295 |
| Prepayments to vendors | 280 | 465 |
| Leasehold deposit | 1,234 | 892 |
| **Total non-current prepayments and deposits** | **2,014** | **1,652** |

Non-current prepayments and deposits mainly includes a deposit with a CRO for advance payment of pass-through costs in connection with a clinical trial, prepaid insurance, and the lease deposit on our headquarters in Copenhagen.

Current prepayments and other receivables are specified below:

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Prepayments to vendors | 38,281 | 13,355 |
| Grant income receivable | 81 | 357 |
| VAT receivable, net | 10,333 | 2,521 |
| Foreign VAT receivable | 1,304 | 1,304 |
| Other current receivables | 1,236 | 1,600 |
| **Total current prepayments and other receivables** | **51,235** | **19,137** |

Current prepayments to vendors include prepayments made to CROs for clinical trial costs of DKK 5.2 million (2019: DKK 5.0 million).

## 3.5 PRE-LAUNCH INVENTORY

### § ACCOUNTING POLICIES

The Company may scale-up and make commercial quantities of its product candidate prior to the date it anticipates that such product will receive final regulatory approval. The scale-up and commercial production of pre-launch inventory involves the risk that such products may not be approved for marketing on a timely basis, or ever. This risk notwithstanding, the Company may scale-up and build pre-launch inventory of product that have not yet received final regulatory approval when the Company believes that such action is appropriate in relation to the commercial value of the product launch opportunity.

Inventory manufactured prior to regulatory approval (pre-launch inventory) is capitalized but immediately provided for, until regulatory approval for the product is obtained. A write-down is made against inventory, and the cost is recognized in the statement of profit and loss and comprehensive income as research and development costs. Once regulatory approval is obtained, the write-down is reversed, up to no more than the original cost.

**Pre-launch inventory intended for commercial sale**

As of December 31, 2020 and 2019, the Company did not have pre-launch inventory that qualified for capitalization. As of December 31, 2020, the Company had pre-launch inventory of approximately DKK 12.4m (2019: DKK 0) intended for commercial sale following regulatory approval of arimoclomol for the treatment of Niemann-Pick Disease Type C (NPC). This amount is fully provided for and recognized under research and development expenses in the statement of profit or loss and other comprehensive loss along with the production costs for drug substance and drug products used in clinical trials and early access programs that are not eligible for reversal at a later time. The provision for pre-launch inventory intended for commercial sale following regulatory approval in the U.S. will be reversed once regulatory approval is obtained.

## 3.6 FINANCIAL ASSETS AND LIABILITIES

### § ACCOUNTING POLICIES

**Financial assets**

*Initial recognition and measurement*

Financial assets that meet certain criteria are classified at initial recognition as subsequently measured at amortized cost, fair value through other comprehensive income (OCI), or fair value through profit or loss. The Group does not hold any financial assets meeting these classification criteria except cash and certain types of other receivables, which are valued at amortized cost. Generally, the Company's financial assets are available to support current operations and amounts expected to be realized within the next twelve months are classified in the Statement of Financial Position as current assets.

The Group's financial assets are recognized initially at fair value plus, in the case of financial assets not carried at fair value through profit and loss, transaction costs that are attributable to the acquisition of the financial asset, if any. Financial instruments recognized at fair value are allocated to one of the following valuation hierarchy levels:

- Level 1: Quoted (unadjusted) prices in active markets for identical assets or liabilities.
- Level 2: Other techniques for which all inputs that have a significant effect on the recorded fair value are observable, either directly or indirectly.
- Level 3: Techniques that use inputs that have a significant effect on the recorded fair value that are not based on observable market data.

*Subsequent measurement*

Historically, the Group's receivables are due within a twelve-month period and therefore the impact of using the effective interest rate method on the Group's financial statements has been immaterial.

*Financial asset impairment*

The Group assesses at the end of each reporting period whether there has been objective evidence that a financial asset may be impaired. Impairment losses are recognized if there is objective evidence of impairment and the evidence indicates that estimated future cash flows will be negatively impacted. The Group did not assess an impairment of a financial asset for either of the years ended December 31, 2020 or 2019.

**Financial liabilities**

*Borrowings*

Financial liabilities, including borrowings, are initially measured at fair value less transaction costs incurred. Subsequently, borrowings are measured at amortized cost. Amortized cost is calculated as original cost less instalments plus/less the accumulated amortization of the difference between cost and nominal value, so that the effective interest rate is recognized in the income

## 3.6 FINANCIAL ASSETS AND LIABILITIES (CONTINUED)

statement over the loan period. Financial liabilities are derecognized when settled.

The Facilitation Fee in our Loan Agreement, defined below, is accounted for as an embedded derivative. The variability arising from the change in Orphazyme's share price is not closely related to the host debt instrument characterized mainly by interest rate and credit risk. Therefore, the embedded equity-linked amount is separated from the host debt instrument and accounted for as an embedded written call option at fair value through profit and loss.

The portion of the debt maturing after one year is presented as non-current debt and the remainder as current debt.

*Trade payables and accruals*
Trade payables and accruals relate to the Group's purchase of products and services from various vendors in the normal course of business.

*Other liabilities*
Other payables are measured at amortized cost. The amount payable to employees for the Phantom Shares Program (Note 2.5) is classified as non-current and is measured at fair value, at Level 2 in the fair value hierarchy.

The Group's financial assets include mainly cash (Note 3.7). The Group has no derivative financial assets nor has there been a change in classification of a financial asset after initial recognition and measurements as discussed herein. Financial assets are not acquired for trading or speculative purposes, nor has the Group placed any assets as security for loans at either December 31, 2020 or 2019.

**Estimate of accruals related to clinical trial development costs**
As explained in Note 2.1, Orphazyme incurs substantial costs associated with clinical trials related to its development programs and there is a high degree of estimation involved in accounting for clinical trial development costs. As described in Note 2.1, Management uses an expense model to estimate the timing of expenses recognition in each period and related accruals at the end of the year.

The Group's financial liabilities comprise the following as of the years ended December 31:

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Borrowings | 57,180 | 62,824 |
| Lease liabilities (Note 3.2) | 13,534 | 12,689 |
| Trade payables | 29,937 | 1,093 |
| Accruals | 42,198 | 31,297 |
| **Total liabilities measured at amortized cost** | **142,849** | **107,903** |

As of the year ended December 31, 2020, Accruals includes an amount of DKK 12.3 million (2019: DKK 13.3 million) for clinical trial costs.

**Kreos Debt Facility**
In August 2019, Orphazyme entered into a structured debt facility ("Loan Agreement") with Kreos Capital to secure funding of €9 million (Tranche 1") to be repaid over forty-two months ("Loan Term"), with the first twelve months requiring interest only payments at nominal annual fixed interest rate of 9.75% and the remaining thirty months requiring equal installments comprising principal and interest. Early repayment of the borrowed amounts may be made in whole but not in part, with the repayment amount being equal to the principal outstanding plus the sum of all the interest repayments that would have been paid throughout the remainder of the loan discounted at an annual rate of 4.0%.

In addition, the lender may, at any time in its sole discretion in eight years, depending on certain events defined in the Loan Agreement, notify the Company that a Facilitation Fee is due and payable ("Notification").

The Facilitation Fee is an amount equal to the greater of (i) 10% of the aggregate amount of the amount borrowed and (ii) the percentage increase in the Company's share price on Nasdaq Copenhagen between the 30-day volume-weighted average share price on the date of the Loan Agreement and the closing share price on the day immediately preceding the date of the notification applied to the aggregate amount of amounts borrowed. The variability arising from the change in Orphazyme's share price is not closely related to the host debt instrument characterized mainly by interest rate and credit risk. Therefore, the embedded equity-linked amount is separated from the host debt instrument and accounted for as an embedded written call option at fair value through profit and loss.

Fair value on inception of the Loan Agreement is included as part of the transaction costs. The call option is measured at fair value at level 2 in the fair value hierarchy.

The written call option is measured at fair value using a Black-Scholes option valuation model. In measuring the fair value, various observable and unobservable inputs are required. Observable input mainly relates to the market price of Orphazyme's shares, and risk-free interest rate. Unobservable inputs mainly relate to the expected volatility of Orphazyme's share price, which was determined based on the Company's own historical volatility, and the term. The table below shows the inputs used in the valuation of the call option and the estimated fair value at the date of the Loan Agreement and on December 31:

## 3.6 FINANCIAL ASSETS AND LIABILITIES (CONTINUED)

| CALL OPTION ON FACILITATION FEE | Dec 2020 | Dec 2019 |
|---|---|---|
| Fair value of call option | 838 | 1,595 |
| Dividend yield (%) | - | - |
| Expected volatility (%) | 54% | 57% |
| Risk-free interest rate (%) | (0.61)% | (0.63)% |
| Expected life (years) | 2.2 | 3.2 |
| Share price (DKK) | 67.1 | 72.4 |

The change in fair value of the call option is recognized as a finance income or expense in the statement of profit or loss. For the year ended December 31, 2020, the Company recognized a gain of DKK 0.8 million (2019: a loss of DKK 0.4 million).

The structured debt facility included a potential second tranche available to Orphazyme, however as of December 31, 2019 conditions allowing for the drawdown of the second tranche were not met and it expired unused. In connection with the drawdown of Tranche 1, Orphazyme incurred transaction costs in the amount of €0.5 million (DKK 3.4 million). As the transaction costs secured a potential financing of two tranches, half of the transaction costs, or €0.2 million (DKK 1.7 million) are being amortized with the first tranche and upon expiration of the second tranche, the other half of the transaction costs were written off as finance expense in the statement of profit or loss (Note 2.6).

As part of the closing of the Loan Agreement, Orphazyme made a payment in the amount of €0.4 million (DKK 2.5 million) as a deposit for the last cash payment to be made on the borrowing ("Advance Payment").

The total liability for the Loan Agreement is being amortized net of the transaction costs, the Facilitation Fee and the call option; and it is being presented net of the Advance Payment.

*Maturities of financial liabilities*

The table below presents the Group's financial liabilities by relevant maturity groupings based on their contractual maturities for all non-derivative financial liabilities and derivative financial instruments for which the contractual maturities are essential for an understanding of the timing of the cash flows.

As the Facilitation Fee is due upon demand, it is shown as current Borrowings under non-derivatives. The call option on the Facilitation Fee is shown as current under derivatives.

The amounts disclosed in the table are the contractual undiscounted cash flows. Balances due within 12 months equal their carrying balances as the impact of discounting is not significant.

| (DKK 000) | Less than 12 months | Between 1 and 2 years | Between 2 and 5 years | Total contractual cash flows | Carrying amount |
|---|---|---|---|---|---|
| **Non-derivatives** | | | | | |
| Trade payables and accruals | 72,135 | — | — | 72,135 | 72,135 |
| Borrowings | 30,126 | 30,126 | 4,524 | 64,776 | 57,179 |
| Lease liabilities | 4,323 | 4,297 | 6,418 | 15,038 | 13,534 |
| **Total non-derivatives** | **106,584** | **34,423** | **10,942** | **151,949** | **142,848** |
| Derivatives (Borrowings) | 838 | | | 838 | 838 |
| **Total derivatives** | **838** | | | **838** | **838** |

| | | Non-cash changes | | | | |
|---|---|---|---|---|---|---|
| (DKK 000) | Dec 31, 2019 | Cash flow | Additions | Adjustments and modifications | Accumulated interest | Exchange-rate adjustments | Dec 31, 2020 |
| Borrowings | 62,824 | (16,349) | - | 750 | 9,921 | 33 | 57,179 |
| Lease liabilities | 12,689 | (3,678) | 3,963 | - | 567 | (7) | 13,534 |
| **Total liabilities from financing activities** | **75,513** | **(20,027)** | **3,963** | **750** | **10,488** | **26** | **70,713** |

| | | Non-cash changes | | | | |
|---|---|---|---|---|---|---|
| (DKK 000) | Dec 31, 2018 | Cash flow | Additions | Adjustments and modifications | Accumulated interest | Exchange-rate adjustments | Dec 31, 2019 |
| Borrowings | 61,543 | (2,185) | - | (1,324) | 4,917 | (127) | 62,824 |
| Lease liabilities | - | (3,838) | 17,014 | (838) | 351 | - | 12,689 |
| **Total liabilities from financing activities** | **61,543** | **(6,023)** | **17,014** | **(2,162)** | **5,268** | **(127)** | **75,513** |

## 3.6 FINANCIAL ASSETS AND LIABILITIES (CONTINUED)

Total current other liabilities are comprised of the following as of the years ended December 31:

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Deferred grant income | - | 95 |
| Remuneration to the Board of Directors | 2,840 | 1,535 |
| Payroll and employee-related costs | 50,487 | 16,279 |
| **Total current other liabilities** | **53,327** | **17,909** |

In addition, the Group has the following total other non-current liabilities as of the years ended December 31:

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Accrual for milestone payment to vendor | 1,179 | 65 |
| Phantom shares liability to employees | 455 | 313 |
| **Total non-current other liabilities** | **1,634** | **378** |

## 3.7 CASH

### § ACCOUNTING POLICIES

Cash includes cash on hand and in banks. Please see Financial Risks discussed in Note 4.4.

The Group's cash balance denominated in foreign currencies were as follows as of the years ended December 31:

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| DKK | 483,862 | 89,155 |
| USD | 241,353 | 14,309 |
| EUR | 644 | 20,083 |
| CHF | 18 | - |
| GBP | 1,052 | 41 |
| **Total cash** | **726,929** | **123,588** |

## 3.8 COMMITMENTS AND CONTINGENCIES

**Contractual commitments for the acquisition of intangible assets**

As disclosed in Note 3.1, under the terms of the Asset Purchase Agreement with CytRx, the Company agreed to make future payments to CytRx that were contingent upon the achievement of specified clinical, regulatory, and sales milestones. The Company has agreed to pay CytRx clinical and regulatory milestone payments for the first two product candidates being developed or labeled for indications other than for the treatment or prevention of ALS or stroke (non-ALS or stroke products). Payments are triggered upon achieving certain key clinical or regulatory milestones. In 2016, the Company paid CytRx USD 0.1 million for achievement of a clinical milestone for the first product candidate. The maximum aggregate amount of milestone payments that may be triggered is USD 12.1 million for the first non-ALS or stroke product and USD 10.3 million for the second non-ALS or stroke product developed assuming (for both products) approval in the European Union (or certain major European markets), United States and Japan. A second non-ALS or stroke product is not considered a second product (and does not trigger milestone payments) unless it contains a different compound than the first non-ALS or stroke product.

The Company has also agreed to pay CytRx clinical and regulatory milestone payments (payable one time only) for each product candidate developed that is being developed or labelled for the treatment or prevention of ALS or stroke (ALS or stroke products). Payments are triggered upon achieving certain key clinical or regulatory milestones. In August 2018, the Company made a milestone payment of USD 250,000 (DKK 1.6 million) upon the enrollment of the first patient in the ALS Phase 3 clinical trial. The maximum aggregate amount of milestone payments that may be triggered per ALS or stroke product is USD 23.8 million assuming approval in the European Union (or certain major European markets), the United States and Japan. The milestone obligations are payable only once per ALS or stroke product. A subsequent ALS or stroke product may achieve an additional maximum aggregate amount of USD 23.8 million in milestone payments, only if it contains a different compound than an ALS or stroke product previously achieving same milestone but is for a different indication.

The Asset Purchase Agreement further includes sales milestones and royalty payments to be made by the Company based on a specified percentage of any eventual net sales of products containing one of the compounds purchased. In addition, under the terms of the Asset Purchase Agreement, the Company was assigned and became party to a royalty agreement with ALS Charitable Remainder Trust pursuant to which the Company is obliged to pay a 1% royalty to the ALS Charitable Remainder Trust on worldwide net sales of arimoclomol for the treatment of ALS.

The first sales milestone is triggered on aggregated annual global net sales exceeding USD 100 million. The aggregate milestone payment obligation may be up to USD 50 million assuming aggregated annual global net sales in excess of USD 1 billion. The Company has agreed to pay CytRx a low teens double-digit royalty on net sales of all products developed by the Company, its affiliates or licensees which are labeled or prescribed for the treatment or prevention of ALS or stroke and a mid-single digit royalty on net sales of all other products developed by the Company or its affiliates or licensees containing any of the compounds purchased from CytRx. Royalties accrue on a country-by-country and product-by-product basis until the latest of expiration of relevant patent claims in the country covering such product, expiry of regulatory exclusivity in the country for such product or ten years from the date of the approval of the product in the country. The royalty rates are subject to reductions for patent expiration, lack of regulatory exclusivity, third party payments and generic competition.

The Group has entered into an operating lease for its headquarters location in Denmark. The lease component portion is accounted for under IFRS 16 (see Note 3.2) and the non-lease component portion, which consists of basic services and maintenance, has a non-cancellable lease term of six months. At December 31, 2020, the non-lease component established a contractual commitment of DKK 1.5 million (2019: DKK 0.5 million).

**Contractual obligations toward CROs**

In addition, the Group has contractual obligations related to contracts with CROs and other vendors for research and development activities that have been initiated and are non-cancelable as of December 31, 2020. These establish contractual commitments of approximately DKK 192 million (2019: DKK 178 million).

**Pledges and securities for loans**

In connection with a loan agreement in the amount up to €18.0 million entered into on August 27, 2019 with Kreos Capital VI (UK) Ltd., the Company has granted security in favor of Kreos Capital VI (UK) Ltd. over (i) certain of its assets, including its intellectual property rights, pursuant to a floating charge agreement registered with the Danish personal register in the initial principal amount of €9.0 million, (ii) its patents registered in Germany, the UK and the US pursuant to a patent pledge agreement and (iii) its shares in its US subsidiary, Orphazyme US, Inc. Furthermore, Orphazyme US, Inc. has granted in favor of Kreos Capital VI (UK) Ltd. (i) a guarantee for the Company's obligations under the loan agreement pursuant to a guaranty agreement and (ii)

## 3.8 COMMITMENTS AND CONTINGENCIES (CONTINUED)

security over certain of its assets, including its intellectual property rights, pursuant to a security agreement governed under US law.

**Contingent asset: Priority review voucher.** The U.S. FDA has granted Orphazyme a rare pediatric disease designation to arimoclomol as a treatment for Niemann-Pick disease Type C (NPC). Under the FDA's rare pediatric disease priority review voucher program, upon the approval of a new drug application for the treatment of a rare pediatric disease, the sponsor of such application would be eligible for a rare pediatric disease priority review voucher that can be used to obtain priority review for a subsequent new drug application. However, receiving a rare pediatric disease designation for arimoclomol as a treatment for NPC does not automatically mean that the Company will receive a priority review voucher as priority review voucher is only awarded following approval by the FDA of arimoclomol as a treatment for NPC. If a priority review voucher is granted, the Company may use the voucher for its own FDA approval processes or decide to sell the voucher to other biotech or pharmaceutical companies. There is no established market for priority review vouchers and disclosed sales prices may not be indicative of the current value of vouchers, which may also fluctuate significantly. If a drug candidate receives Rare Pediatric Disease Designation before September 20, 2024, it is eligible to receive a voucher if it is approved before September 30, 2026. Hence, it may be unavailable to the Company even if it meets all of the requirements. Further, the potential award of a voucher would trigger an obligation to market the relevant rare pediatric disease product within one year from FDA approval or the FDA may revoke the voucher. Finally, a voucher award subjects the Company to post marketing reporting obligations to the FDA.

SECTION 4

# OTHER DISCLOSURES

**Section 4 presents details of other disclosures relevant to the consolidated financial statements of the Group, including Capital Management, Equity, Earnings per Share, Financial Risk, Related Parties, Fees to Statutory Auditors, and Significant Events after the Reporting Period.**

**In this section**


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## 4.1 CAPITAL MANAGEMENT

For the purpose of the Group's capital management, capital includes issued capital, share premium and all other equity reserves attributable to the equity holders of the Group. The primary objective of the Group's capital management is to maximize shareholder value while limiting the financial risk. The Board of Directors' policy is to maintain a strong capital base in order to maintain investor, creditor and market confidence, and a continuous advancement of the Group's intellectual property, product pipeline, and business.

The Company strengthened its cash position during 2020 by raising net proceeds of DKK 694.2 million from a directed issue and private placement in February; and in September by listing American Depositary Shares (ADSs) on the Nasdaq Global Select Market, whereby we raised net proceeds of DKK 477.9 million.

As of December 31, 2020, the Group held cash totaling DKK 726.9 million (2019: DKK 123.6 million). Management monitors the Company's funding risks and liquidity needs, with a view to continue as a going concern. Subsequently, funding options are evaluated on a continuous basis, including public or private debt and equity financing in order to ensure sufficient funding to continue ongoing clinical trials and commercial launch activities. Should the Company find itself unable to attain funding, the Company may down-size or delay planned activities to allow funds to last until December 31, 2021. Management therefore considers it appropriate to prepare these financial statements on a going concern basis.

## 4.2 EQUITY

The following table summarizes the Company's share activity:

| | Ordinary shares |
|---|---|
| **December 31, 2017** | **19,928,184** |
| Issuance of bonus shares as part of license agreement (note 3.1) | 11,380 |
| **December 31, 2018** | **19,939,564** |
| Issuance of bonus shares as part of license agreement (note 3.1) | 26,060 |
| Issuance of Matching Shares (Note 2.6) | 19,175 |
| **December 31, 2019** | **19,984,799** |
| Issuance of bonus shares as part of license agreement (note 3.1) | 20,650 |
| Issuance of Matching Shares (Note 2.6) | 31,250 |
| Issuance of shares due to exercise of restricted share-units | 11,921 |
| Issuance of shares related to directed issue and private placement, February 2020 | 7,032,937 |
| Issuance of shares related to US listing, September 2020 | 7,616,146 |
| **December 31, 2020** | **34,697,703** |

The Company has never declared or paid any cash dividends on its ordinary shares and does not anticipate doing so in the foreseeable future. The Company intends to use all available financial resources as well as revenue, if any, for purposes of the Company's current and future business.

In March 2019, the Company issued 19,175 Matching Shares to participants in the 2017 LTIP (see Note 2.5)

In January 2020, the Company issued 20,650 (2019: 26,060) bonus shares to KLSDC and UCL under the terms of the license agreement discussed in Note 3.1.

In February 2020, the Company completed an offering of 7,032,937 shares in a directed issue and private placement and raised gross proceeds of approximately DKK 745 million and net proceeds of approximately DKK 694 million.

The transaction consisted of a directed issue and private placement of up to 3,961,264 new shares of a nominal value of DKK 1 each (the "New Shares") and private placement of up to 3,071,673 existing shares of a nominal value of DKK 1 each (the "Existing Shares" and together with the New Shares, the "Offer Shares") at an offer price of DKK 106 per Offer Share, as determined by the Board of Directors of the Company through a book-building process (the "Offering"). The New Shares will be issued without pre-emption rights for existing shareholders.

The offering of Existing Shares was facilitated by a share loan from Novo Holdings A/S and Orpha Pooling B.V. (the "Lending Shareholders") to the Company pursuant to a stock lending and subscription agreement with an obligation for the Company to redeliver new shares of an equivalent number as the Existing Shares borrowed by the Company from each of the Lending Shareholders (the "Replacement Shares"), which were issued without pre-emption rights for existing shareholders. The Lending Shareholders did not participate in the Offering and only facilitated the loan of the Lending Shares for purposes of the Company's offering of Existing Shares in the Offering.

In April 2020, the Company issued 5,378 new shares to board members following the exercise of fully vested RSUs under the 2019 RSU program (see Note 2.5).

In July 2020, the Company issued 31,250 Matching Shares to participants in the 2019 LTIP (see Note 2.5)

In September 2020, the Company listed American Depositary Shares (ADSs) on the Nasdaq Global Select Market. In connection with this listing, we issued and sold 3,650,000 ordinary shares and 3,966,146 ADSs, each representing one ordinary share. Aggregate gross proceeds from the offering amounted to USD 87.8 million (DKK 534.5 million). Orphazyme incurred transaction costs in the amount of DKK 56.6 million in connection with the US listing, which were accounted for as a deduction from equity.

As a result of the above transactions, the total nominal share capital of the Company as of December 31, 2020 was DKK 34,697,703, divided into 34,697,703 ordinary shares each with a nominal value of DKK 1.

## 4.3 LOSS PER SHARE

Basic loss per share for the year is calculated by dividing the net loss for the year by the weighted average number of ordinary shares outstanding during the year. The diluted loss per share is calculated by dividing the net loss for the year by the weighted average number of ordinary shares outstanding during the period increased by the dilutive effect of the assumed issuance of outstanding share-based awards. As a result of the Group incurring losses for each of the years ended December 31, 2020, 2019 and 2018, the potential shares issuable related to outstanding share-based awards have been excluded from the calculation of diluted per share amounts, as the effect of such shares is anti-dilutive.

Basic and diluted loss per share for the years presented have been adjusted retrospectively to include the 2018 Bonus Shares, the 2019 Bonus Shares and the 2020 Bonus Shares discussed in Note 3.1 in the number of weighted average shares outstanding for the years ended December 31, 2020, 2019, and 2018. This results in the comparative figures for 2019 and 2018 being updated accordingly.

Basic and diluted loss per share for the years presented have been adjusted retrospectively to include the 2019 Bonus Shares, the 2020 Bonus Shares and the 2021 Bonus Shares in the number of weighted average shares outstanding for the years ended December 31, 2020, 2019 and 2018. This results in the comparative figure for 2019 and 2018 being updated accordingly.

The following reflects the net loss attributable to shareholders and share data used in the basic and diluted earnings/(loss) per share computations for the years ended December 31:

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| Net loss for the year (DKK 000) | (633,246) | (337,497) | (229,600) |
| Weighted-average shares outstanding | 28,366,469 | 20,024,692 | 20,008,827 |
| **Loss per share** | **(22.32)** | **(16.85)** | **(11.47)** |

## 4.4 FINANCIAL RISKS

The Group's activities expose it to a number of financial risks whereby future events, which can be outside the control of the Group, could have a material effect on its financial position and results of operations. The known risks include foreign currency, interest and credit risk and there could be other risks currently unknown to Management. The Group has not historically hedged its financial risks.

**Liquidity Risk**
At December 31, 2020, the Group's liquidity risk was assessed to be high. Management continuously assesses the Group's capital structure in order to evaluate whether its liquidity reserves allow it to achieve its business objectives. At December 31, 2020, the available liquidity reserves were assessed to be sufficient to provide adequate funding to allow the Group to meet its planned operating activities, including increased levels of research and development activities, in the normal course of business for the next twelve months.

**Foreign Currency Risk**
The Group's foreign currency risk is assessed to be high. The Group conducts cross border transactions where the functional currency of the respective group entity is not always used. Accordingly, future changes in the exchange rates of the DKK against the EUR, the USD, the CHF and/or the GBP will expose the Group to currency gains or losses that will impact the reported amounts of assets, liabilities, income and expenses and the impact could be material.

**Interest Rate Risk**
The Group's interest rate risk is assessed to be low. The Group has a borrowing on which it incurs a fixed rate of interest (see Note 3.6). In addition, due to the current interest level in Denmark, the Group incurs negative interest on bank deposits.

**Credit Risk**
The Group's credit risk is assessed to be low. The Group's credit risk is associated with cash held in banks. The Company does not trade financial assets for speculative purposes and invests with the objective of preserving capital. The Company's cash is held primarily at two banks in Denmark with Moody's long-term credit ratings exceeding of A1.

## 4.4 FINANCIAL RISKS (CONTINUED)

The Group has prepared a sensitivity analysis in order to assess the potential impact on the Group's net loss for possible fluctuations in the EUR and USD exchange rates against the DKK and the impact for the possible fluctuations in the interest rate on bank deposits in Denmark and in the USA. The methods and assumptions used are consistent with prior year and consider increases and decreases in the Group's three main currencies, as well as reasonable fluctuations in the interest rate on its bank deposits. Based on these analyses, if interest rates on our cash deposits would have fluctuated by +/- 1%, the impact on the Group's net loss for the year ended December 31, 2020 would have been approximately DKK 36 thousand (2019: DKK 8 thousand; 2018: DKK 20 thousand).

The impact of currency fluctuations on the Group's net loss is shown in the table below:

| CURRENCY | Currency fluctuation | Effect 2020 TDKK | Effect 2019 TDKK | Effect 2018 TDKK |
|---|---|---|---|---|
| EUR | +/- 2% | 538 | 503 | 713 |
| USD | +/-10% | 22,178 | 21 | 1,278 |
| CHF | +/-10% | 611 | - | - |
| GBP | +/-10% | 199 | 461 | 218 |

## 4.5 REMUNERATION OF BOARD OF DIRECTORS AND EXECUTIVE MANAGEMENT

Executive Management consists of the Company's Chief Executive Officer and the Chief Financial Officer, also the registered management of the Company. In July 2019, Orphazyme announced that the Board of Directors appointed Kim Stratton as the new Chief Executive Officer, succeeding Anders Hinsby on October 1, 2019.

Ms. Stratton resigned from her position at Orphazyme on December 10, 2020. As part of the separation agreement, Ms. Stratton will continue to receive her monthly base salary during 2021 and on December 31, 2021 she will receive severance pay equal to one year's base salary. Therefore as of December 31, 2020, two times her base salary is additionally recognized as salary expense. Subsequent to December 31, 2020, Ms. Stratton received 35,304 Matching Shares as part of the 2020 LTIP program and will receive 58,000 ordinary shares as part of the sign-on bonus described in Note 2.5. As of December 31, 2020, 52,956 Performance Shares had vested as part of the 2020 LTIP program. These awards will be settled in January 2024 based on the development of the Company's share price (Note 2.5).
As of December 31, 2020, the position of Chief Executive Officer was vacant.

The Executive Management is eligible to receive an annual performance-based cash bonus subject to certain predefined corporate and individual goals as determined by the Board of Directors on an annual basis. A cash bonus received under the short-term incentive program may not exceed 100% of the annual fixed salary of the participants. As part of the separation agreement, Ms. Stratton is entitled to the annual performance-based cash bonus. The Executive Management is also eligible to receive an extraordinary bonus at the discretion of the Board of Directors.

# 4.5 REMUNERATION OF BOARD OF DIRECTORS AND EXECUTIVE MANAGEMENT (CONTINUED)

## REMUNERATION TO INDIVIDUAL MEMBERS OF EXECUTIVE MANAGEMENT

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| **Anders Hinsby (former CEO)** | | | |
| Salary | - | 2,424 | 1,917 |
| Bonus | - | 1,038 | 723 |
| Share-based compensation | - | 294 | 676 |
| Other employee benefits | - | 270 | 215 |
| **Total** | **-** | **4,026** | **3,531** |
| | | | |
| **Kim Stratton (CEO through December 31, 2020)** | | | |
| Salary* | 11,001 | 962 | - |
| Bonus | 3,500 | 1,025 | - |
| Share-based compensation | 7,359 | - | - |
| Other employee benefits | 2,542 | 215 | - |
| **Total** | **24,402** | **2,202** | **-** |
| | | | |
| **Anders Vadsholt (Interim CEO and CFO)** | | | |
| Salary | 2,324 | 1,803 | 1,411 |
| Bonus | 2,491 | 1,250 | 450 |
| Share-based compensation | 2,805 | 406 | 460 |
| Other employee benefits** | 983 | 260 | 161 |
| **Total** | **8,603** | **3,719** | **2,485** |
| | | | |
| **Total remuneration to the Executive Management** | **33,005** | **9,947** | **6,016** |

* Includes two times annual base salary as per separation agreement
** Includes holiday allowance in the amount of DKK 0.7 million that is accounted for as a benefit to Mr. Vadsholt but will not be paid out to him

Remuneration paid to members of the Board of Directors is made up of board and committee fees, a travel allowance, ad-hoc fees for additional services provided as described in Note 4.6, and share-based compensation related to the Restricted Share Units (RSUs) as described in Note 2.5.

Board remuneration is recognized as general and administrative expenses in the Statement of Profit or Loss. The following table lists Board of Directors remuneration for the years ended December 31:

## REMUNERATION TO INDIVIDUAL MEMBERS OF THE BOARD OF DIRECTORS

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| **Georges Gemayel (Chairman of the Board)** | | | |
| Board and committee fees | 565 | 470 | 468 |
| Ad hoc board fees | 186 | - | - |
| Travel allowance | 27 | 64 | 47 |
| Share-based compensation | 161 | 28 | - |
| **Total** | **939** | **562** | **515** |
| | | | |
| **Bo Jesper Hansen (Deputy Chairman of the Board)** | | | |
| Board and committee fees | 421 | 395 | 394 |
| Ad hoc board fees | 112 | - | - |
| Travel allowance | 34 | 46 | 33 |
| Share-based compensation | 102 | 21 | - |
| **Total** | **669** | **462** | **427** |
| | | | |
| **Martin Bonde** | | | |
| Board and committee fees | 276 | 259 | 258 |
| Travel allowance | - | - | - |
| Share-based compensation | 73 | 16 | - |
| **Total** | **349** | **275** | **258** |
| | | | |
| **Martijn Kleijwegt** | | | |
| Board and committee fees | 304 | 285 | 284 |
| Travel allowance | 35 | 46 | 33 |
| Share-based compensation | 73 | 16 | - |
| **Total** | **412** | **347** | **317** |

## 4.5 REMUNERATION OF BOARD OF DIRECTORS AND EXECUTIVE MANAGEMENT (CONTINUED)

| (DKK 000) | 2020 | 2019 | 2018 |
|---|---|---|---|
| **Rémi Droller** | | | |
| Board and committee fees | 288 | 270 | 269 |
| Travel allowance | 25 | 46 | 33 |
| Share-based compensation | 73 | 16 | - |
| **Total** | **386** | **332** | **302** |
| | | | |
| **Sten Verland** | | | |
| Board and committee fees | 327 | 309 | 307 |
| Travel allowance | 1 | - | - |
| Share-based compensation | 73 | 16 | - |
| **Total** | **401** | **325** | **307** |
| | | | |
| **Anders Hedegaard** | | | |
| Board and committee fees | 288 | 270 | 269 |
| Travel allowance | 13 | 46 | - |
| Share-based compensation | 73 | 16 | - |
| **Total** | **374** | **332** | **269** |
| | | | |
| **Catherine Moukheibir** | | | |
| Board and committee fees | 355 | 336 | 335 |
| Ad hoc boord fees | 112 | - | - |
| Travel allowance | 23 | 46 | 33 |
| Share-based compensation | 73 | 16 | - |
| **Total** | **563** | **398** | **368** |
| | | | |
| **Carrolee Barlow** | | | |
| Board and committee fees | 77 | - | - |
| Travel allowance | - | - | - |
| Share-based compensation | 145 | - | - |
| **Total** | **222** | **-** | **-** |
| | | | |
| **Total remuneration to the Board of Directors** | **4,315** | **3,033** | **2,763** |

## 4.6 RELATED PARTIES

Orphazyme A/S, incorporated in Denmark, is the ultimate parent company of the Group, which wholly owns Orphazyme US, Inc and Orphazyme Switzerland GmbH. These three entities are considered related parties. Orphazyme A/S is not ultimately controlled by any of its investors. Major investors owning more than 10% of the Company are considered related parties.

For the years ended December 31, 2020, 2019, and 2018 the following related party transactions were identified:

- Remuneration to Executive Management (Note 4.5)
- Remuneration to the Board of Directors (Note 4.5)
- Participation of Executive Management in the 2017 LTIP, the 2019 LTIP and the 2020 LTIP (Note 4.5)
- Participation of the Board members in the RSU, program (Note 4.5)
- Share lending arrangement in connection with the directed issue and private placement in February 2020 (Note 4.2). We entered into a Stock Lending and Subscription Agreement on February 6, 2020 with Danske Bank A/S, Orpha Pooling B.V. and Novo Holdings A/S, pursuant to which we borrowed 3,071,673 existing ordinary shares (the Lending Shares) from Orpha Pooling B.V. and Novo Holdings A/S, major investors at the time, through Danske Bank A/S as settlement agent in order for us to place such ordinary shares in a private placement. The Lending Shares were borrowed subject to an obligation for us to issue new ordinary shares of an equivalent number as the Lending Shares placed in this private placement, or the Listing Shares, and for Danske Bank A/S to use the proceeds from the sale of Lending Shares in the private placement to subscribe for the Listing Shares and deliver the Listing Shares to the Orpha Pooling B.V. and Novo Holdings A/S. The Listing Shares were issued and delivered, as agreed, on February 11, 2020
- Ad-hoc fees paid to certain members of the Board of Directors in connection for their support during the US listing process. Total ad-hoc fees amounted to EUR 55,000 (DKK 0.4 million).
- Ad-hoc fees paid to the Chairman of the Board in connection with a consultancy agreement for support during the interim period until a new CEO is hired. As part of this agreement, Orphazyme has paid the Chairman of the Board an up-front payment of EUR 88,605 (DKK 0.7 million) in December 2020. An additional payment of 100% of his aggregate annual board and committee fees is payable in June 2021. In addition to cash compensation, the Chairman has been granted 4.351 RSUs under the 2020-2 RSU program (see Note 3.5). The full remuneration to the Chairman of the Board under this consultancy agreement is subject to the approval of the shareholders at the Company's annual general meeting in March 2021.

As of December 31, 2020 and 2019, the Company did not have any amounts receivable from related parties and therefore recorded no related impairment. The Company has not granted any loans, guarantees, or other commitments to or on behalf of any of the members of the Board of Directors or Executive Management. For amounts payable to the Board of Directors, please see Note 3.6.

Executive Management and members of the Board of Directors had the following shareholding in Orphazyme A/S for the years ended December 31:

| | Number of shares owned 2020 | Number of shares owned 2019 | Number of shares owned 2018 |
|---|---|---|---|
| Kim Stratton | 50,600 | - | - |
| Anders Vadsholt | 143,156 | 132,595 | 127,806 |
| Anders Hinsby (Former CEO) | - | 209,596 | 204,596 |

| | December 31, 2020 | | | December 31, 2019 | | December 31, 2018 |
|---|---|---|---|---|---|---|
| MEMBERS OF THE BOARD OF DIRECTORS: | Number of shares owned | Number of Unvested RSUs 2020 | Number of Unexercised RSUs 2019 | Number of shares owned | Number of Unvested RSUs 2019 | Number of shares owned |
| Georges Gemayel | 100,809 | 4,351 | - | 97,358 | 3,451 | 87,758 |
| Bo Jesper Hansen | 143,234 | 2,689 | - | 100,545 | 2,689 | 79,945 |
| Martijn Kleijwegt | - | 1,927 | 1,927 | - | 1,927 | - |
| Martin Bonde | 47,936 | 1,927 | - | 46,009 | 1,927 | 46,009 |
| Rémi Droller | - | 1,927 | 1,927 | - | 1,927 | - |
| Sten Verland | - | 1,927 | - | - | 1,927 | - |
| Anders Hedegaard | 15,677 | 1,927 | - | 13,750 | 1,927 | 6,250 |
| Catherine Moukheibir | 7.980 | 1,927 | 1,927 | 7,980 | 1,927 | 7,980 |
| Carrolee Barlow | - | 4,391 | - | - | - | - |

## 4.7 FEES TO STATUTORY AUDITORS

The following table presents the fees to our independent statutory auditors, EY Godkendt Revisionspartnerselskab (formerly Ernst & Young P/S), recognized in general and administrative expenses in the Statement of Profit or Loss for the years ended December 31. This note has been prepared in accordance with the requirements in the Danish Financial Statements Act.

| (TDKK) | 2020 | 2019 | 2018 |
|---|---|---|---|
| Audit services | 2,416 | 2,244 | 320 |
| Audit-related services | 803 | 882 | 156 |
| Other assistance | 3,795 | - | 50 |
| **Total fees to statutory auditors** | **7,014** | **3,126** | **526** |

**Audit services**
Audit services consist of fees billed for professional services rendered by EY for the audit of our annual consolidated financial statements or services that are normally provided by the accountant in connection with statutory and regulatory filings or engagements for those fiscal years.

**Audit-Related services**
Audit-related services consist of assurance and related services performed by EY that are reasonably related to the performance of the audit or review of our consolidated financial statements and are not reported under "Audit services".

**Other assistance**
Other assistance consists of services provided by EY for other permitted services, including fees for work performed by EY in connection with the U.S. listing in September 2020.

**Pre-approval policies**
The Audit Committee assesses and pre-approves all services provided by the statutory auditors. The pre-approval includes the type of service and a fee budget.

## 4.8 SIGNIFICANT EVENTS AFTER THE REPORTING PERIOD

In March 2021, Christophe Bourdon was appointed Chief Executive Officer, effective April 1, 2021.

In January 2021, the Matching Shares part of the 2020 LTIP program fully vested. As a result, 170,131 ordinary shares were issued to Executive Management and other employees of Orphazyme against receipt of the nominal value of DKK 1 per ordinary share. In addition, subsequent to year-end 58,000 ordinary shares were issued to our former CEO, Kim Stratton (see Note 4.5).

As described in Note 3.1, as part of the license agreement with KLSDC and UCL, consideration to KLSDC and UCL is payable in shares of the Company ("Bonus Shares") each January and is based on incurred costs reported by KLSDC and UCL for the previous year. As at December 31, 2020 the aggregate costs incurred by KLSDC and UCL amounted to USD 0.5 million (DKK 3.2 million) representing a total of 41,109 Bonus Shares ("2020 Bonus Shares"), may be issued to KLSDC and UCL in Q1-2021 based on the average 30-day closing price of Orphazyme's shares. As an alternative to the share issuance, the cash equivalent of the value of the Bonus Shares may be paid instead. As elected by the respective parties, KLSDC will receive 22,553 shares and UCL will receive an amount of USD 267,076 (DKK 1,636,869).

# 2020
# Parent Company Financial Statements

# STATEMENTS OF PROFIT OR LOSS AND OTHER COMPREHENSIVE INCOME

## PARENT COMPANY

| FOR THE YEARS ENDED DECEMBER 31 (DKK 000) | Note | 2020 | 2019 |
|---|---|---|---|
| Research and development expenses | 2.1 | (362,413) | (285,413) |
| General and administrative expenses | 2.2 | (258,933) | (49,682) |
| **Operating expenses** | | **(621,346)** | **(335,095)** |
| Financial income | 2.4 | 2,427 | 316 |
| Financial expenses | 2.4 | (28,985) | (7,359) |
| **Net loss before tax** | | **(647,905)** | **(342,138)** |
| Income tax benefit | 2.5 | 5,500 | 5,500 |
| **Net loss for the year** | | **(642,405)** | **(336,638)** |
| *Items that will be reclassified subsequently to the Statement of Profit or Loss:* | | | |
| Exchange difference from translation of foreign operations | | (54) | - |
| **Total comprehensive income** | | **(642,459)** | **(336,638)** |

# STATEMENTS OF FINANCIAL POSITION

**PARENT COMPANY, AS OF DECEMBER 31**

| ASSETS (DKK 000) | Note | 2020 | 2019 |
|---|---|---|---|
| **Non-current assets** | | | |
| Right-of-use assets | 2.7 | 12,862 | 13,903 |
| Intangible assets | 2.6 | 10,681 | 10,539 |
| Property, plant, and equipment | 2.8 | 3,707 | 3,685 |
| Investment in subsidiaries | 2.9 | 139 | - |
| Prepayments and deposits | 2.10 | 1,666 | 1,652 |
| Corporation tax receivable | 2.5 | 2,750 | 2,750 |
| **Total non-currents assets** | | **31,805** | **32,529** |
| **Current assets** | | | |
| Corporation tax receivable | 2.5 | 5,500 | 5,500 |
| Prepayments and other receivables | 2.10 | 262,173 | 18,957 |
| Cash | 2.12 | 487,322 | 123,250 |
| **Total current assets** | | **754,995** | **147,707** |
| **TOTAL ASSETS** | | **786,800** | **180,236** |

| EQUITY AND LIABILITIES (DKK 000) | Note | 2020 | 2019 |
|---|---|---|---|
| Share capital | | 34,698 | 19,984 |
| Share premium | | 2,082,255 | 924,021 |
| Other reserves | | 5,674 | 7,822 |
| Accumulated deficit | | (1,511,221) | (898,159) |
| **Total shareholders' equity** | | **611,406** | **53,668** |
| **Non-current liabilities** | | | |
| Borrowings | 2.11 | 23,830 | 51,611 |
| Lease liabilities | 2.7 | 8,071 | 9,813 |
| Other non-current liabilities* | | 1,634 | 378 |
| **Total non-current liabilities** | | **33,535** | **61,802** |
| **Current liabilities** | | | |
| Current borrowings | 2.11 | 33,349 | 12,808 |
| Lease liabilities | 2.7 | 3,276 | 2,876 |
| Trade payables and accruals | 2.11 | 49,074 | 31,173 |
| Other liabilities | 2.11 | 56,160 | 17,909 |
| **Total current liabilities** | | **141,859** | **64,766** |
| **Total liabilities** | | **175,394** | **126,568** |
| **TOTAL SHAREHOLDERS' EQUITY AND LIABILITIES** | | **786,800** | **180,236** |

# STATEMENTS OF CHANGES IN SHAREHOLDERS' EQUITY

## PARENT COMPANY

| (DKK 000) | Note | Share capital | Share premium | Foreign currency translation reserve | Share-based compensation - acquisition of intangible assets | Accumulated deficit | Total |
|---|---|---|---|---|---|---|---|
| **Balance as of December 31, 2018** | | **19,939** | **924,021** | **(51)** | **9,070** | **(564,823)** | **388,156** |
| Net loss for the period | | - | - | - | - | (336,638) | (336,638) |
| Other comprehensive loss for the period | | - | - | - | - | - | - |
| Total other comprehensive loss | | - | - | - | - | - | (336,638) |
| *Transactions with owners:* | | | | | | | |
| Issuance of Bonus Shares* | | 26 | - | - | (1,197) | 1,171 | - |
| Issuance of Matching Shares, net of costs* | | 19 | - | - | - | - | 19 |
| Share-based compensation expense* | | - | - | - | - | 2,131 | 2,131 |
| **December 31, 2019** | | **19,984** | **924,021** | **(51)** | **7,873** | **(898,159)** | **53,668** |
| Net loss for the period | | - | - | - | - | (642,405) | (642,405) |
| Other comprehensive loss for the period | | - | - | (54) | - | - | (54) |
| Total other comprehensive loss | | - | - | - | - | - | (642,459) |
| *Transactions with owners*:* | | | | | | | |
| Issuance of Bonus Shares to KLSDC and UCL | | 21 | - | - | (2,094) | 2,073 | - |
| Issuance of Shares due to exercise of RSUs | | 13 | 717 | - | - | - | 730 |
| Issuance of LTIP Matching Shares | | 31 | - | - | - | - | 31 |
| Issuance of new shares in private placement | | 7,033 | 738,458 | - | - | - | 745,491 |
| Transaction costs in connection with private placement | | - | (51,243) | - | - | - | (51,243) |
| Issuance of new shares in U.S. listing | | 7,616 | 526,918 | - | - | - | 534,534 |
| Transaction costs in connection with U.S. listing | | - | (56,616) | - | - | - | (56,616) |
| Share-based payments costs for the period | | - | - | - | - | 27,270 | 27,270 |
| **December 31, 2020** | | **34,698** | **2,082,255** | **(105)** | **5,779** | **(1,511,221)** | **611,406** |

*Please refer to respective notes in the Consolidated Financial Statements of the Group

# STATEMENTS OF CASH FLOWS

## PARENT COMPANY, FOR THE YEARS ENDED DECEMBER 31

| (DKK 000) | Note | 2020 | 2019 |
|---|---|---|---|
| **Net loss** | | (642,405) | (336,638) |
| *Adjustment for non-cash items:* | | | |
| Share-based compensation expenses | 2.3 | 20,823 | 2,550 |
| Depreciation and amortization | 2.6, 2.7, 2.8 | 4,742 | 5,010 |
| Financial income | 2.4 | (2,427) | (316) |
| Financial expenses | 2.4 | 28,985 | 7,359 |
| Income tax benefit, net | 2.5 | (5,500) | (5,500) |
| *Change in working capital:* | | | |
| Change in prepayments, deposits, and other receivables | 2.10 | (32,552) | 5,003 |
| Change in trade payables, accruals, and other liabilities | 2.11 | 49,502 | (2,854) |
| Change in intercompany payables | 2.10 | (211,878) | (825) |
| Change in intercompany receivables | | 7,907 | - |
| Exchange rate adjustments on intercompany balances | | (4,044) | - |
| Interest paid | | (10,639) | (5,181) |
| Interest received | | 28 | 388 |
| Income taxes paid | | - | - |
| Income taxes received | | 5,500 | 5,500 |
| **Cash flow from operating activities** | | **(791,957)** | **707** |
| Investment in intangble assets | 2.6 | (896) | (508) |
| Investment in property, plant, and equipment | 2.8 | (1,259) | (2,777) |
| Proceeds from sale of property, plant, and equipment | | - | - |
| **Cash flow from investing activities** | | **(3,86)** | **(3,285)** |
| Proceeds from issue of shares and exercise of RSUs | | 1,280,786 | 19 |
| Transaction costs | | (107,859) | - |
| Proceeds from borrowings | | - | 62,758 |
| Repayment of borrowings | | (10,535) | - |
| Repayment of leasing liabilities | 2.7 | (2,913) | (3,838) |
| **Cash flow from financing activities** | | **1,159,478** | **58,939** |
| **Changes in cash and cash equivalents** | | **365,366** | **56,361** |
| Cash and cash equivalents at the beginning of the period | | 123,250 | 393,123 |
| Exchange rate adjustments on cash and cash equivalents | | (1,294) | (23) |
| **Cash and cash equivalents at the end of the period** | | **487,322** | **123,250** |

SECTION 1

# BASIS OF PREPARATION

**The financial statements of Orphazyme A/S (the "Parent Company") have been prepared in accordance with International Financial Reporting Standards, or IFRS, as adopted by the EU and additional disclosure requirements under the Danish Financial Statements Act.**

**The Parent Company financial statements of Orphazyme A/S for the year ended December 31, 2020 were approved by the Board of Directors on March 2, 2021 and will be submitted to the shareholders of Orphazyme A/S for approval at the Annual General Meeting to be held on March 25, 2021.**

**In this section**


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| 1.2 | Significant accounting policies applicable to the parent company | 87 |


## 1.1 CORPORATE INFORMATION

In April 2018, a wholly-owned subsidiary, Orphazyme U.S., Inc., was incorporated in Delaware, USA and in March 2020, a wholly-owned subsidiary, Orphazyme Schweiz GmbH, was incorporated in Zug, Switzerland (together with Orphazyme A/S, "Orphazyme" or "the Group"). By establishing local subsidiaries, the Company aims to directly support the U.S. and European markets and establish closer relationships with the medical, patient, and financial communities as Orphazyme expands its development programs and global reach.

## 1.2 SIGNIFICANT ACCOUNTING POLICIES APPLICABLE TO THE PARENT COMPANY

The Parent Company applies the same accounting policies as disclosed in the Group's consolidated financial statements. Therefore, only accounting policies specific to the Parent Company or that differ from the accounting policies applied by the Group are disclosed in these notes to the parent statements. If an accounting policy is not specifically mentioned, the Group accounting policy is applied.

A description of Management's key accounting estimates and judgements as well as new IFRS standards are disclosed in the Group financial statements and also apply to the Parent Company.

The Parent Company financial statements are presented in Danish Kroner, or DKK, which is both the functional and presentation currency of the Parent Company. Where indicated, amounts are rounded to the nearest thousand, or TDKK.

SECTION 2

# NOTES

The notes applicable to the financial statements of the Parent Company are the same as those presented in the Group Consolidated Financial Statements, except for those notes presented in this Section 2.

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## 2.1 RESEARCH AND DEVELOPMENT EXPENSES

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| External costs | 256,445 | 214,766 |
| Intercompany expenses | 8,752 | 5,510 |
| Employee costs (Note 2.3) | 93,261 | 62,034 |
| Depreciation and amortization (Note 3.1, 3.2, 3.3) | 3,955 | 3,103 |
| **Total** | **362,413** | **285,413** |

## 2.2 GENERAL AND ADMINISTRATIVE EXPENSES

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| External costs | 54,251 | 17,860 |
| Intercompany expenses | 139,582 | 9,757 |
| Employee costs (Note 2.3) | 64,313 | 21,366 |
| Depreciation and amortization (Note 3.1, 3.2, 3.3) | 787 | 699 |
| **Total** | **258,933** | **49,682** |

## 2.3 EMPLOYEE COSTS

| EMPLOYEE COSTS (DKK 000) | 2020 | 2019 |
|---|---|---|
| Salaries | 97,444 | 63,075 |
| Cash bonus | 21,636 | 7,991 |
| Share-based compensation costs | 19,976 | 2,405 |
| Pension | 8,914 | 5,515 |
| Other social security contributions | 2,020 | 532 |
| Other staff costs | 3,267 | 849 |
| **Total** | **153,257** | **80,367** |
| Board remuneration | 3,470 | 3,033 |
| Board share-based compensation | 847 | - |
| **Total employee costs** | **157,574** | **83,400** |
| **Recognized as follows in the Statement of Profit or Loss:** | | |
| Research and development expenses | 93,261 | 62,034 |
| General and administrative expenses | 64,313 | 21,366 |
| **Total** | **157,574** | **83,400** |
| Average number of full-time employees | 94 | 74 |
| Year-end number of full-time employees | 102 | 83 |

## 2.4 FINANCIAL INCOME AND FINANCIAL EXPENSES

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Interest income on cash balances | 28 | 316 |
| Foreign currency exchange gains | 1,649 | - |
| Gain on embedded call option (Note 3.6) | 750 | - |
| **Total financial income** | **2,427** | **316** |
| Interest expense on Loan Agreement (Note 3.6) | 9,921 | 3,239 |
| Write-off of transaction costs for Loan Agreement tranche 2 (Note 3.6) | - | 1,678 |
| Loss on embedded call option (Note 3.6) | - | 354 |
| Interest expense on lease liabilities (Note 3.2) | 499 | 351 |
| Loss on lease modification (Note 3.2) | - | 216 |
| Interest expense on cash balances | 3,608 | 1,213 |
| Foreign currency exchange loss | 14,805 | 229 |
| Bank fees and other charges | 152 | 79 |
| **Total financial expenses** | **28,985** | **7,991** |

## 2.5 INCOME TAXES

The following table presents the total income tax benefit for the years ended December 31, 2020 and 2019:

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Current tax benefit on net loss | 142,539 | 75,270 |
| Adjustments prior years | (1,065) | - |
| Tax credit research and development expenses | 5,500 | 5,500 |
| Change in unrecognized deferred tax before tax credit | (144,256) | (74,507) |
| Permanent differences | 2,782 | (763) |
| **Total income tax benefit for the period** | **5,500** | **5,500** |

The following table presents the reconciliation of the effective tax rate to the statutory corporate income tax rate in Denmark.

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Net loss before tax | (647,905) | (342,138) |
| Corporate income tax rate in Denmark | 22% | 22% |
| **Computed income tax benefit** | **142,539** | **75,270** |
| *Tax effect of:* | | |
| Other non-deductible expenses, including | | |
| Adjustments prior years | (1,065) | - |
| IPO-related costs and share-based compensation | 2,782 | 763 |
| Deferred tax asset not recognized after tax credit | (138,756) | (69,007) |
| **Total income tax benefit for the period** | **5,500** | **5,500** |

The following table presents the carrying amount of deferred tax in the Statement of Financial Position:

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Tax deductible losses | 192,837 | 93,030 |
| Deferred tax on intangible assets | 112,192 | 74,050 |
| Other temporary differences | 2,019 | 758 |
| | 307,048 | 167,838 |
| Deferred tax asset not recognized | 307,048 | 167,838 |
| **Carrying amount included on statement of financial position** | **-** | **-** |

At December 31, 2020, Orphazyme had tax receivables of DKK 8,250 thousand(hereof DKK 2,750 thousand as non-current receivables) related to the refundable tax credits for costs incurred in connection with research and development activities under the Danish Tax Credit Regime.

## 2.6 INTANGIBLE ASSETS

(DKK 000)

| 2020 | Software | Licenses | Total |
|---|---|---|---|
| Cost at the beginning of the year | - | 12,083 | 12,083 |
| Additions during the year | 896 | - | 896 |
| Disposals during the year | - | - | - |
| Exchange rate adjustment | - | - | - |
| **Cost at the end of the year** | **896** | **12,083** | **12,979** |
| | | | |
| Depreciation at beginning of the year | - | (1,543) | (1,543) |
| Depreciation for the year | (42) | (712) | (754) |
| Depreciation reversed on disposals during the year | - | - | - |
| Exchange rate adjustment | - | - | - |
| Depreciation at the end of the year | (42) | (2,256) | (2,298) |
| **Carrying amount at the end of the year** | **854** | **9,827** | **10,681** |

| 2019 | Software | Licenses | Total |
|---|---|---|---|
| Cost at the beginning of the year | - | 11,575 | 11,575 |
| Additions during the year | - | 508 | 508 |
| Disposals during the year | - | - | - |
| Exchange rate adjustment | - | - | - |
| **Cost at the end of the year** | **-** | **12,083** | **12,083** |
| | | | |
| Depreciation at beginning of the year | - | (831) | (831) |
| Depreciation for the year | - | (712) | (712) |
| Depreciation reversed on disposals during the year | - | - | - |
| Exchange rate adjustment | - | - | - |
| Depreciation at the end of the year | - | (1,543) | (1,543) |
| **Carrying amount at the end of the year** | - | **10,539** | **10,539** |

Depreciation and impairment losses:

| **Depreciation are included in the income statement as follows:** | 2020 | 2019 |
|---|---|---|
| General and administrative expenses | 712 | 712 |
| General and administrative expenses | 42 | - |
| **Total depreciation** | **754** | **712** |

## 2.7 LEASES

(DKK 000)

| 2020 | Office buildings | Operating equipment | Total |
|---|---|---|---|
| Cost at the beginning of the year | 11,953 | 4,008 | 15,961 |
| Additions during the year | 1,712 | - | 1,712 |
| Disposals during the year | - | - | - |
| Exchange rate adjustment | - | - | - |
| **Cost at the end of the year** | **13,665** | **4,008** | **17,673** |
| | | | |
| Depreciation at beginning of the year | (1,858) | (200) | (2,058) |
| Depreciation for the year | (2,351) | (401) | (2,752) |
| Depreciation reversed on disposals during the year | - | - | - |
| Exchange rate adjustment | - | - | - |
| Depreciation at the end of the year | (4,209) | (601) | (4,810) |
| **Carrying amount at the end of the year** | **9,456** | **3,406** | **12,862** |

| 2019 | | | |
|---|---|---|---|
| Cost at the beginning of the year | 13,006 | - | 13,006 |
| Additions during the year | - | 4,008 | 4,008 |
| Modification during the year | (1,053) | - | (1,053) |
| Exchange rate adjustment | - | - | - |
| **Cost at the end of the year** | **11,953** | **4,008** | **15,961** |
| | | | |
| Depreciation at beginning of the year | - | - | - |
| Depreciation for the year | (1,858) | (200) | (2,058) |
| Depreciation reversed on disposals during the year | - | - | - |
| Exchange rate adjustment | - | - | - |
| Depreciation at the end of the year | (1,858) | (200) | (2,058) |
| **Carrying amount at the end of the year** | **10,095** | **3,807** | **13,903** |

Depreciation and impairment losses:

| **Depreciation are included in the income statement as follows:** | 2020 | 2019 |
|---|---|---|
| General and administrative expenses | 2,441 | 1,825 |
| General and administrative expenses | 311 | 233 |
| **Total depreciation** | **2,752** | **2,058** |

## 2.7 LEASES (CONTINUED)

The following table presents the carrying amounts of lease liabilities and the movements during the period:

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| At January 1, 2019 | 12,689 | 13,006 |
| Additions | 1,712 | 4,008 |
| Accretion of interest | 499 | 351 |
| Payments | (3,554) | (3,838) |
| Exchange rate adjustments | - | - |
| Modifications | - | (838) |
| **At December 31, 2019** | **11,346** | **12,689** |
| Current | 3,275 | 2,876 |
| Non-current | 8,071 | 9,813 |

## 2.8 PROPERTY, PLANT, AND EQUIPMENT

(DKK 000)

| 2020 | Furniture and equipment | Leasehold improve-ments | Total |
|---|---|---|---|
| Cost at the beginning of the year | 5,532 | 2,066 | 7,598 |
| Additions during the year | 1,259 | - | 1,259 |
| Disposals during the year | - | - | - |
| Exchange rate adjustment | - | - | - |
| **Cost at the end of the year** | **6,791** | **2,066** | **8,857** |
| Depreciation at beginning of the year | (3,614) | (300) | (3,914) |
| Depreciation for the year | (919) | (317) | (1,236) |
| Depreciation reversed on disposals during the year | - | | - |
| Exchange rate adjustment | - | - | - |
| Depreciation at the end of the year | (4,533) | (617) | (5,150) |
| **Carrying amount at the end of the year** | **2,258** | **1,449** | **3,707** |

| 2019 | Furniture and equipment | Leasehold improve-ments | Total |
|---|---|---|---|
| Cost at the beginning of the year | 4,419 | 402 | 4,821 |
| Additions during the year | 1,113 | 1,664 | 2,777 |
| Modification during the year | - | - | - |
| Exchange rate adjustment | - | - | - |
| **Cost at the end of the year** | **5,532** | **2,066** | **7,598** |
| Depreciation at beginning of the year | (2,761) | (120) | (2,881) |
| Depreciation for the year | (853) | (180) | (1,033) |
| Depreciation reversed on disposals during the year | - | - | - |
| Exchange rate adjustment | - | - | - |
| Depreciation at the end of the year | (3,614) | (300) | (3,914) |
| **Carrying amount at the end of the year** | **1,918** | **1,766** | **3,684** |

Depreciation and impairment losses:

| **Depreciation are included in the income statement as follows:** | 2020 | 2019 |
|---|---|---|
| General and administrative expenses | 802 | 670 |
| General and administrative expenses | 434 | 363 |
| **Total depreciation** | **1,236** | **1,033** |

## 2.9 INVESTMENT IN GROUP COMPANIES

### § ACCOUNTING POLICIES

Investments in subsidiaries are measured in the Parent Company financial statements at the lower of cost or recoverable amount.

Any distributed dividends are recognized in the income statmement of the Parent Company.

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Cost at January 1 | 1,207 | 1,207 |
| Additions | 139 | - |
| **Cost end of year December 31** | **1,346** | **1,207** |

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Adjustment January 1 | (1,207) | - |
| Impairment | - | (1,207) |
| **End of period December 31** | **(1,207)** | **(1,207)** |

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| **Carrying amount of investment** | **139** | **-** |

| (000) | Registered office | Ownership interest (%) | Share capital | | Equity | Net result |
|---|---|---|---|---|---|---|
| Orphazyme US, Inc. | Delaware, USA | 100% | USD 1 | (USD 000) | 3,406 | 94 |
| Orhazyme GmbH (CH) | Zug, Switzerland | 100% | CHF 20,000 | (CHF 000) | 291 | 53 |

## 2.10 PREPAYMENTS, DEPOSITS, AND OTHER RECEIVABLES

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Deposits with vendors | 296 | 295 |
| Prepayments to vendors | 280 | 465 |
| Leasehold deposit | 1,090 | 892 |
| **Total non-current prepayments and deposits** | **1,666** | **1,652** |

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Prepayments to vendors | 37,342 | 13,174 |
| Grant income receivable | 81 | 357 |
| VAT receivable, net | 10,331 | 2,522 |
| Foreign VAT receivable | 1,304 | 1,304 |
| Receivables intercompany | 211,878 | - |
| Other current receivables | 1,237 | 1,600 |
| **Total current prepayments and other receivables** | **262,173** | **18,957** |

## 2.11 FINANCIAL ASSETS AND LIABILITIES

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Borrowings | 57,180 | 62,824 |
| Lease liabilities (Note 2.7) | 11,346 | 12,689 |
| Trade payables | 18,376 | 1,093 |
| Accruals | 30,698 | 30,080 |
| **Total liabilities measured at amortized cost** | **117,600** | **106,686** |

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| Deferred grant income | - | 95 |
| Remuneration to the Board of Directors | 2,840 | 1,535 |
| Payables to group entities | 7,907 | - |
| Payroll and employee related costs | 45,413 | 16,279 |
| **Total other current liabilities** | **56,160** | **17,909** |

| | | | Non-cash changes | | | | |
|---|---|---|---|---|---|---|---|
| (DKK 000) | Dec 31, 2019 | Cash flow | Additions | Adjustments and modifications | Acculated interest | Exchange adjustments | Dec 31, 2020 |
| Borrowings | 62,824 | (16,349) | - | 750 | 9,921 | 33 | 57,179 |
| Lease liabilities | 12,689 | (3,554) | 1,712 | - | 499 | - | 11,346 |
| **Total liabilities from financing activities** | **75,513** | **(19,903)** | **1,712** | **750** | **10,420** | **33** | **68,525** |

| | | | Non-cash changes | | | | |
|---|---|---|---|---|---|---|---|
| (DKK 000) | Dec 31, 2018 | Cash flow | Additions | Adjustments and modifications | Acculated interest | Exchange adjustments | Dec 31, 2019 |
| Borrowings | 61,543 | (2,185) | - | (1,324) | 4,917 | (127) | 62,824 |
| Lease liabilities | - | (3,838) | 17,014 | (838) | 351 | - | 12,689 |
| **Total liabilities from financing activities** | **61,543** | **(6,023)** | **17,014** | **(2,162)** | **5,268** | **(127)** | **75,513** |

## 2.12 CASH

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| DKK | 483,872 | 89,153 |
| EUR | 644 | 20,083 |
| USD | 1,765 | 13,972 |
| GBP | 1,051 | 42 |
| **Total cash** | **487,332** | **123,250** |

## 2.13 RELATED PARTY DISCLOSURES

Orphazyme A/S' related parties are the parent company's Board of Directors, Executive Management and close members of the family of these persons.

**Transactions with subsidiaries**
Orphazyme US, Inc. and Orphazyme GmbH (CH) are 100% owned subsidiaries of Orphazyme A/S and are included in the consolidated financial statements. They perform certain research and development, general and administrative and management activities on behalf on the parent company. All intercompany transactions have been eliminated in the consolidated financial statements of the Orphazyme Group.

| (DKK 000) | 2020 | 2019 |
|---|---|---|
| **Transaction with subsidiaries** | | |
| Service fee costs | 148,334 | 15,267 |
| **Balances with subsidiaries** | | |
| Current receivables | 211,878 | - |
| Current payables | 7,907 | - |

Please refer to note 4.6 in the consolidated financial statements for additional information regarding transactions with related parties of the Group.

# 2020
# Statements and Signatures

## STATEMENT BY THE BOARD OF DIRECTORS AND EXECUTIVE MANAGEMENT

The Board of Directors and Executive Management have today considered and approved the Annual Report of Orphazyme A/S for the financial year January 1-December 31, 2020.

The consolidated financial statements of the Group and the Parent Company's financial statements have been prepared in accordance with International Financial Reporting Standards (IFRS) as issued by the International Accounting Standards Board (IASB) and in accordance with IFRS as endorsed by the EU as well as additional disclosure requirements under the Danish Financial Statements Act.

In our opinion, the Group's consolidated financial statements and the Parent Company financial statements provide a fair presentation of the assets, liabilities, and financial position at December 31, 2020 and of the results of the Group's and Parent Company's operations and cash flows for the financial year January 1–December 31, 2020.

In our opinion, Management's Review provides a fair presentation of the development in the Group's operations and financial circumstances, the results of the year, and the overall financial position of the Group as well as a description of the most significant risks and elements of uncertainty facing the Group.

We recommend that the Annual Report be adopted at the Annual General Meeting on March 25, 2021.

Copenhagen, March 2, 2021

Approved at the AGM 25/3/2021

Chairman of the annual general meeting

### BOARD OF DIRECTORS

Georges Gemayel
Georges Gemayel
Mar 4, 2021 8:24 AM EST

**Georges Gemayel**
Chairman of the Board

Martin Bonde
Martin Bonde
Mar 4, 2021 11:51 AM CET

**Martin Bonde**

Rémi Droller
Rémi Droller
Mar 4, 2021 11:20 AM CET

**Rémi Droller**

Sten Verland
Sten Verland
Mar 4, 2021 12:09 PM CET

**Sten Verland**

Carrolee Barlow
Carrolee Barlow
Mar 4, 2021 9:35 AM PST

**Carrolee Barlow**

Bo Jesper Hansen
Mar 4, 2021 11:59 AM CET

**Bo Jesper Hansen**
Deputy Chairman of the Board

Anders Hedegaard
Anders Hedegaard
Mar 4, 2021 3:20 PM CET

**Anders Hedegaard**

Martijn Kleijwegt
Martijn Kleijwegt
Mar 10, 2021 11:46 AM CET

**Martijn Kleijwegt**

Catherine Moukheibir
Catherine Moukheibir
Mar 5, 2021 11:46 AM CET

**Catherine Moukheibir**

### EXECUTIVE MANAGEMENT

Anders Vadsholt
Mar 4, 2021 11:25 AM CET

**Anders Vadsholt**
Interim Chief Executive Officer
and Chief Financial Officer

# INDEPENDENT AUDITOR'S REPORT

## TO THE SHAREHOLDERS OF ORPHAZYME A/S

**Opinion**
We have audited the consolidated financial statements and the parent company financial statements of Orphazyme A/S for the financial year 1 January – 31 December 2020, which comprise statement of profit or loss and other comprehensive income, statement of financial position, statement of changes in shareholders' equity, statement of cash flow and notes, including accounting policies, for the Group and the Parent Company. The consolidated financial statements and the parent company financial statements are prepared in accordance with International Financial Reporting Standards (IFRS) as issued by the International Accounting Standards Board (IASB) and in accordance with IFRS as endorsed by the EU as well as additional disclosure requirements under the Danish Financial Statements Act.

In our opinion, the consolidated financial statements and the parent company financial statements give a true and fair view of the financial position of the Group and the Parent Company at 31 December 2020 and of the results of the Group's and the Parent Company's operations and cash flows for the financial year 1 January – 31 December 2020 in accordance with International Financial Reporting Standards (IFRS) as issued by the International Accounting Standards Board (IASB) and in accordance with IFRS as endorsed by the EU as well as additional disclosure requirements under the Danish Financial Statements Act.

Our opinion is consistent with our long-form audit report to the Audit Committee and the Board of Directors.

**Basis for opinion**
We conducted our audit in accordance with International Standards on Auditing (ISAs) and additional requirements applicable in Denmark. Our responsibilities under those standards and requirements are further described in the "Auditor's responsibilities for the audit of the consolidated financial statements and the parent company financial statements" (hereinafter collectively referred to as "the financial statements") section of our report. We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our opinion.

***Independence***
We are independent of the Group in accordance with the International Ethics Standards Board for Accountants' Code of Ethics for Professional Accountants (IESBA Code) and additional requirements applicable in Denmark, and we have fulfilled our other ethical responsibilities in accordance with these rules and requirements.

To the best of our knowledge, we have not provided any prohibited non-audit services as described in article 5(1) of Regulation (EU) no. 537/2014.

***Appointment of auditor***
We were initially appointed as auditor of Orphazyme A/S on 4 December 2015 for the financial year 1 July to 31 December 2015. We have been reappointed annually by resolution of the general meeting for a total consecutive period of 6 years up until the financial year 2020.

**Key audit matters**
Key audit matters are those matters that, in our professional judgement, were of most significance in our audit of the financial statements for the financial year 2020. These matters were addressed during our audit of the financial statements as a whole and in forming our opinion thereon. We do not provide a separate opinion on these matters. For each matter below, our description of how our audit addressed the matter is provided in that context.

We have fulfilled our responsibilities described in the "Auditor's responsibilities for the audit of the financial statements" section, including in relation to the key audit matters below. Accordingly, our audit included the design and performance of procedures to respond to our assessment of the risks of material misstatement of the financial statements. The results of our audit procedures, including the procedures performed to address the matters below, provide the basis for our audit opinion on the financial statements.

**Statement on the Management's review**
Management is responsible for the Management's review.

Our opinion on the financial statements does not cover the Management's review, and we do not express any form of assurance conclusion thereon.

| KEY AUDIT MATTERS | HOW OUR AUDIT ADDRESSED THE KEY AUDIT MATTER |
|---|---|
| **Accrual for costs incurred through Clinical Research Organisations (CROs)**<br>Orphazyme has entered into several clinical research contracts with Clinical Research Organizations (CROs) that render research and development services to the company. Due to the materiality and complexity of these arrangements management is required to make significant estimates when accounting for the costs associated with research and development services received, including recognition of prepayments, accruals and research and development costs.<br><br>Management estimates duration of trials based on the agreed trial protocols and estimates total cost per treatment per patient based on quotations from the CROs. Management's assumptions in respect of timing on patient enrolment is based on information provided by the CROs.<br><br>Refer to Note 2.1, 3.4 and 3.6 to the Consolidated Financial Statements | We have obtained an understanding of the process for accounting for costs associated with research and development services received and evaluated the design of the internal controls relating to the recognition and measurement of such costs.<br><br>For example, we have performed walk-through of management's controls over review of CRO contracts, controls over reconciliation of estimation assumptions to third party confirmations from CROs, controls over timing of patient enrolment, controls over estimated duration of the clinical trial and estimated costs per treatment per patient.<br><br>To test management's estimation models, we obtained management's models for accounting for research and development services rendered by CROs to the company, supporting the accounting for CRO accruals, related prepayments and research and development costs.<br><br>We have validated the key assumptions and data applied by management by comparing total contract costs to specific terms in the individual CRO contracts, total expected patients in the study protocol, patients enrolled and length of the study to clinical trial protocols, historical experience with the CROs and comparing management's estimation models to industry guidance for accounting for clinical trials.<br><br>We further examined subsequent settlement of obligations recognized as at January 1, 2020 and December 31, 2020 to assess completeness and accuracy of the recorded accruals, prepayment and research and development costs. We performed an independent assessment of the most significant elements of the accruals and prepayments as of December 31, 2020 and compared these to the actual accruals and prepayments recognized.<br><br>Further we tested management's models for clerical accuracy. |

In connection with our audit of the financial statements, our responsibility is to read the Management's review and, in doing so, consider whether the Management's review is materially inconsistent with the financial statements or our knowledge obtained during the audit, or otherwise appears to be materially misstated.

Moreover, it is our responsibility to consider whether the Management's review provides the information required under the Danish Financial Statements Act.

Based on the work we have performed, we conclude that the Management's review is in accordance with the financial statements and has been prepared in accordance with the requirements of the Danish Financial Statements Act. We did not identify any material misstatement of the Management's review.

**Management's responsibilities for the financial statements**

Management is responsible for the preparation of consolidated financial statements and parent company financial statements that give a true and fair view in accordance with International Financial Reporting Standards (IFRS) as issued by the International Accounting Standards Board (IASB) and in accordance with IFRS as endorsed by the EU as well as additional disclosure requirements under the Danish Financial Statements Act and for such internal control as Management determines is necessary to enable the preparation of financial statements that are free from material misstatement, whether due to fraud or error.

In preparing the financial statements, Management is responsible for assessing the Group's and the Parent Company's ability to continue as a going concern, disclosing, as applicable, matters related to going concern and using the going concern basis of accounting in preparing the financial statements unless Management either intends to liquidate the Group or the Parent Company or to cease operations, or has no realistic alternative but to do so.

**Auditor's responsibilities for the audit of the financial statements**

Our objectives are to obtain reasonable assurance as to whether the financial statements as a whole are free from material misstatement, whether due to fraud or error, and to issue an auditor's report that includes our opinion. Reasonable assurance is a high level of assurance, but is not a guarantee that an audit conducted in accordance with ISAs and additional requirements applicable in Denmark will always detect a material misstatement when it exists. Misstatements can arise from fraud or error and are considered material if, individually or in the aggregate, they could reasonably be expected to influence the economic decisions of users taken on the basis of the financial statements.

As part of an audit conducted in accordance with ISAs and additional requirements applicable in Denmark, we exercise professional judgement and maintain professional scepticism throughout the audit. We also:

- Identify and assess the risks of material misstatement of the financial statements, whether due to fraud or error, design and perform audit procedures responsive to those risks and obtain audit evidence that is sufficient and appropriate to provide a basis for our opinion. The risk of not detecting a material misstatement resulting from fraud is higher than for one resulting from error, as fraud may involve collusion, forgery, intentional omissions, misrepresentations or the override of internal control.

- Obtain an understanding of internal control relevant to the audit in order to design audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Group's and the Parent Company's internal control.
- Evaluate the appropriateness of accounting policies used and the reasonableness of accounting estimates and related disclosures made by Management.

- Conclude on the appropriateness of Management's use of the going concern basis of accounting in preparing the financial statements and, based on the audit evidence obtained, whether a material uncertainty exists related to events or conditions that may cast significant doubt on the Group's and the Parent Company's ability to continue as a going concern. If we conclude that a material uncertainty exists, we are required to draw attention in our auditor's report to the related disclosures in the financial statements or, if such disclosures are inadequate, to modify our opinion. Our conclusions are based on the audit evidence obtained up to the date of our auditor's report. However, future events or conditions may cause the Group and the Parent Company to cease to continue as a going concern.

- Evaluate the overall presentation, structure and contents of the financial statements, including the note disclosures, and whether the financial statements represent the underlying transactions and events in a manner that gives a true and fair view.

- Obtain sufficient appropriate audit evidence regarding the financial information of the entities or business activities within the Group to express an opinion on the consolidated financial statements. We are responsible for the direction, supervision and performance of the group audit. We remain solely responsible for our audit opinion.

We communicate with those charged with governance regarding, among other matters, the planned scope and timing of the audit and significant audit findings, including any significant deficiencies in internal control that we identify during our audit.

We also provide those charged with governance with a statement that we have complied with relevant ethical requirements regarding independence, and to communicate with them all relationships and other matters that may reasonably be thought to bear on our independence, and where applicable, related safeguards.

From the matters communicated with those charged with governance, we determine those matters that were of most significance in the audit of the consolidated financial statements and the parent company financial statements of the current period and are therefore the key audit matters. We describe these matters in our auditor's report unless law or regulation precludes public disclosure about the matter or when, in extremely rare circumstances, we determine that a matter should not be communicated in our report because the adverse consequences of doing so would reasonably be expected to outweigh the public interest benefits of such communication.

Copenhagen, 2 March 2021
EY GODKENDT
REVISIONSPARTNERSELSKAB
CVR no. 30 70 02 28

**Christian Schwenn Johansen**
State Authorised
Public Accountant
MNE no.: mne33234

DocuSigned by:
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**Anders Roe Eriksen**
State Authorised
Public Accountant
MNE no.: mne46667

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Anders Roe Eriksen
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