Orphazyme

H1 2020 Results

August 28, 2020

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Important notice

This presentation contains forward-looking statements that involve risks and uncertainties. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on our current expectations and projections about future events and financial trends that we believe may affect our financial condition, results of operations, business strategy, and financial needs. All statements other than statements of historical facts contained in this presentation, including any statements regarding the ability of our clinical trials to demonstrate acceptable safety and efficacy of our product candidate, and other positive results; the timing, progress and results of clinical trials for our product candidate, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, the period during which the results of the trials will become available, and our research and development programs; the timing, scope and likelihood of regulatory filings, NDA submissions and approvals, including the rolling submission of a NDA process for arimoclomol for the treatment of NPC and final regulatory approval of arimoclomol; our ability to obtain marketing approvals of our product candidate and to meet existing or future regulatory standards or comply with post-approval requirements; our expectations regarding the potential market size and the size of the patient populations for our product candidate, if approved for commercial use; our expectations regarding the potential advantages of our product candidate over existing therapies; the impact of COVID-19 on our business and operations; our potential to enter into new collaborations; our expectations with regard to our ability to develop additional product candidates or product candidates for other indications our ability to identify additional products, product candidates or technologies with significant commercial potential that are consistent with our commercial objectives; our ability to develop, acquire and advance additional product candidates into, and successfully complete, clinical trials; the initiation, timing, progress and results of our preclinical studies and clinical trials, and our research and development programs; the commercialization and market acceptance of our product candidate; our marketing and manufacturing capabilities; the pricing of and reimbursement for our product candidate; the implementation of our business model and strategic plans for our business and product candidate; regulatory development in the United States, Europe and other jurisdictions; our ability to effectively manage our anticipated growth; our financial performance and projections relating to our competitors and our industry, including competing therapies are forward-looking statements. The words "may," "will," "expect," "anticipate," "aim," "estimate," "intend," "plan," "believe," "is/are likely to," "potential," "continue" and other similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

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Business Review

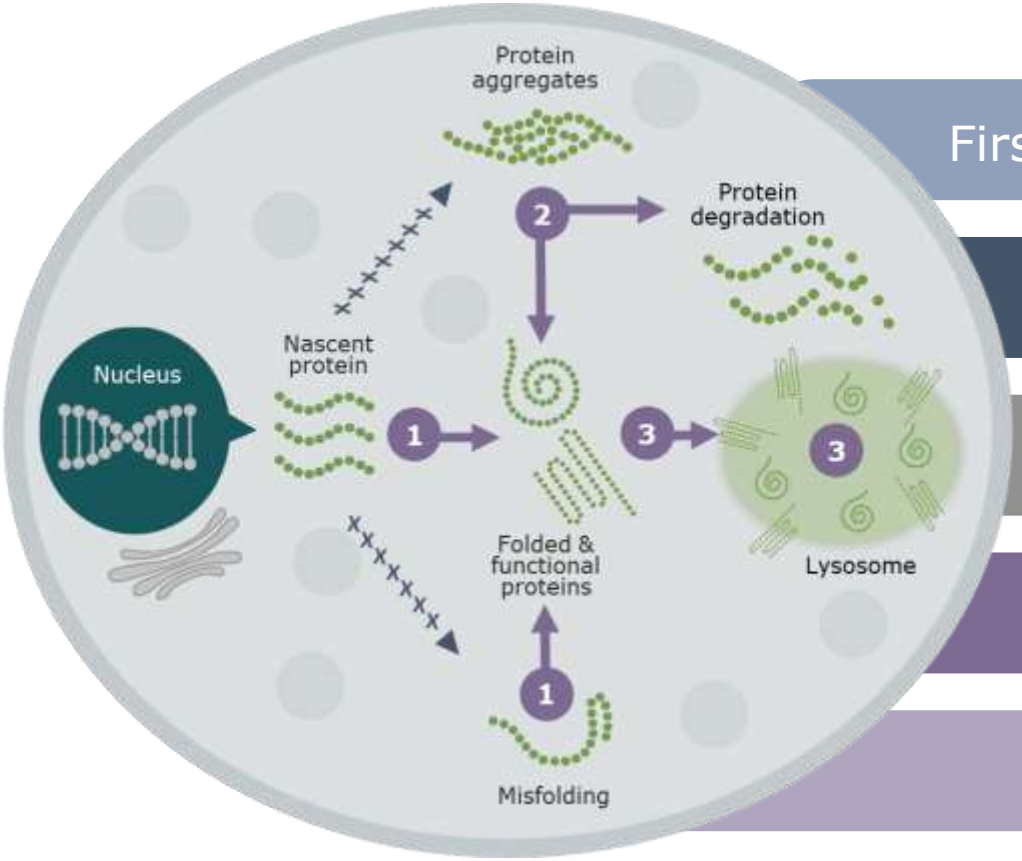
Kim Stratton,
Chief Executive Officer

H1 2020 Achievements: Significant progress on key priorities

Advancing arimoclomol in NPC and beyond	• Positive data from Ph 2/3 12-month open-label extension study in NPC • US Early Access Program in NPC initiated (1st patient treated July) • Initiated rolling submission of NDA with US FDA (completed July 2020) • Positive data on clinical endpoints in Ph 2 Gaucher disease trial (liver & spleen size) • US Fast Track Designation granted in Amyotrophic Lateral Sclerosis • Joined The Michael J. Fox Foundation's Parkinson's Disease RTC[1] (July 2020)
Preparing for launch	• Hired key personnel to continue to build highly specialized commercial sales organization in US and EU in anticipation of potential approval in NPC
Corporate & financial	• Further strengthened balance sheet and expanded shareholder base with directed share issue and private placement raising ~DKK745 million (~USD 110 million)

1. Research Tools Consortium

Arimoclomol: Potential treatment for orphan neurodegenerative diseases



First-in-class^ heat shock protein (HSP) amplifier

The heat shock response is the body's natural defense to acute cell stress

A small molecule that crosses the blood brain barrier

Exposure in ~500 individuals with no significant safety concerns identified

Easy oral or naso-gastric administration

^ First clinical product candidate to harness this mechanism of action for the treatment of lysosomal storage diseases and neuromuscular diseases affecting the central nervous system and muscle. Figure shows key HSP70 effects: 1. chaperone nascent and misfolded proteins, ensuring correct folding and function; 2. dissolve protein aggregates, potentially restoring folded and functional proteins, or ensuring removal by facilitating degradation; 3. promote lysosomal function and stabilize lysosomal membrane, preventing cell death.

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Arimoclomol: Pipeline-in-a-product potential

Rare Pediatric Disease Designation (RPD) in NPC

	Designations			Stage of Development				
Lysosomal storage diseases	Orphan Drug	Fast Track	BTD†	PC	Ph 1	Ph 2	Ph 3	**Key milestones**
Niemann-Pick disease Type C^	✓	✓	✓	Ph 2/3 (data reported)				Rolling NDA submission completed 07/2020; submit MAA (EU) H2 2020
Neurological Gaucher disease				Ph 2‡ (top-line data reported)				
Neuromuscular disorders								
Amyotrophic Lateral Sclerosis	✓	✓		Ph 3 (registrational)				Top-line results H1 2021
Sporadic Inclusion Body Myositis	✓	✓		Ph 2/3 (registrational)				Top-line results H1 2021

^Early access program in US underway; † Breakthrough Therapy Designation; ‡ Type 1 and Type 3 Gaucher disease

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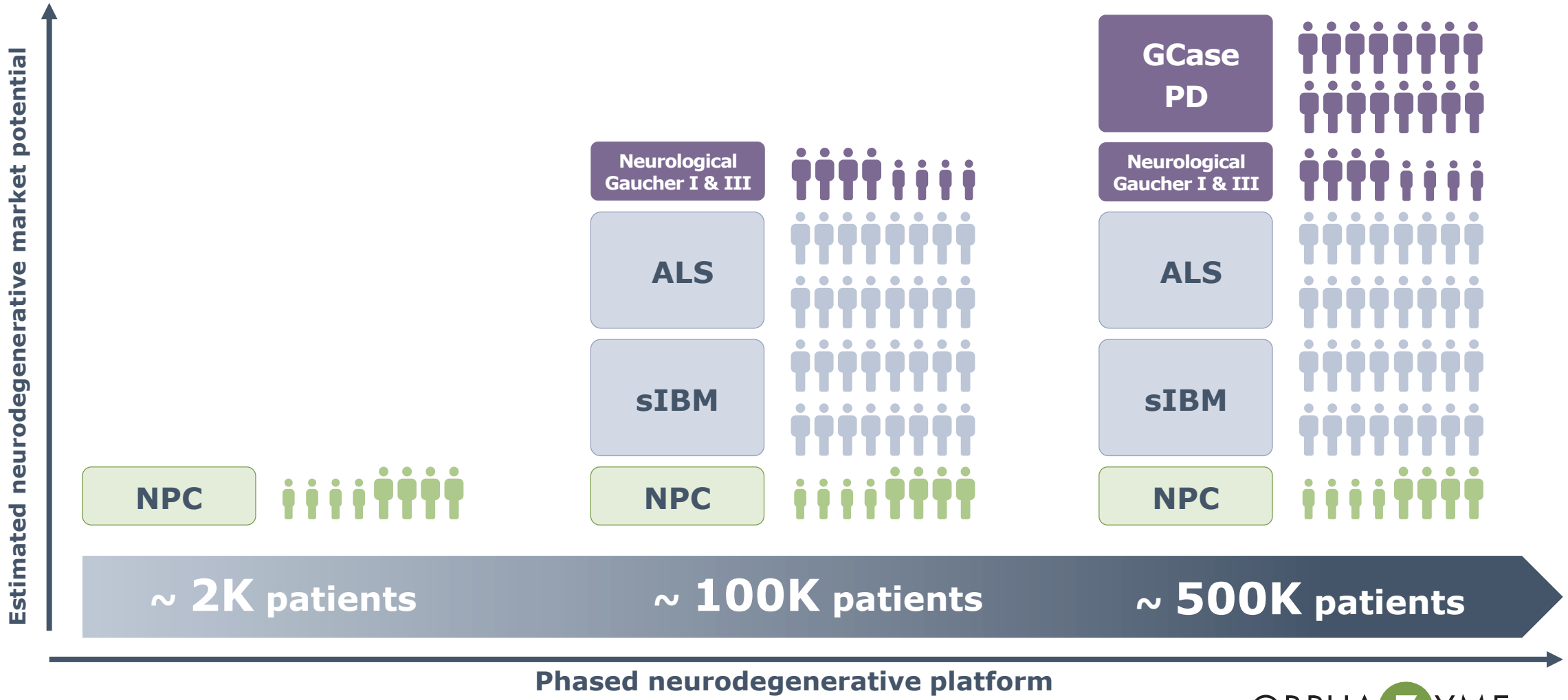
Unlocking significant potential in neurodegenerative orphan diseases



Notes: Estimated patients represent US and EU combined and figures are rounded.

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NPC: a devastating ultra-rare genetic disorder, life-limiting with high unmet need

Substantial impact on patient's and their family's lives

- Progressive lysosomal storage disorder
- Affects ambulation, fine motor skills, swallowing, cognition, speech
- Frequently fatal by the time patients reach their twenties
- Adult-onset NPC increasingly diagnosed; ~50% of diagnosed patients

Lack of treatment options

- US: no approved therapy
- EU: generic miglustat only

Attractive ultra-orphan market

- ~1,800 patients US & EU, of which ~1,100 diagnosed
- Managed in ~ 25 – 50 highly specialized centers

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Enabling patient access and preparing for a potential US Launch

Market Readiness

- NDA submitted (July ’20)
- Payer research on value communications and messaging
- Distribution/ specialty pharmacy partnerships established

Stakeholder Education

- Relationships with specialist centers
- US Steering Committees
- Medical education program

Organizational Readiness

- Highly experienced team
- Leadership & Head Office roles filled
- Recruitment of MSLs/ Field Market Access and KAMs underway

Patient Access & Initiatives

- Early Access Program enrolling
- Conferences and awareness initiatives

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Objective to seek approval and enable access for patients in EU and ROW

Market Readiness

- MAA submission planned H2'20
- Market access plan developed
- Leverage US/EU authorisations

Stakeholder Education

- Advisory Board in Q3 2020
- Payer Panel in Q3 2020
- Digital awareness initiatives

Organizational Readiness

- Appointing General Managers
- Supply chain strategy defined
- Rare disease expert partners to reach RoW patients

Patient Access & Initiatives

- Exploring EAPs in other countries
- Conferences and awareness initiatives
- May explore EAPs where possible in RoW

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Financial Review

Anders Vadsholt,
Chief Financial Officer

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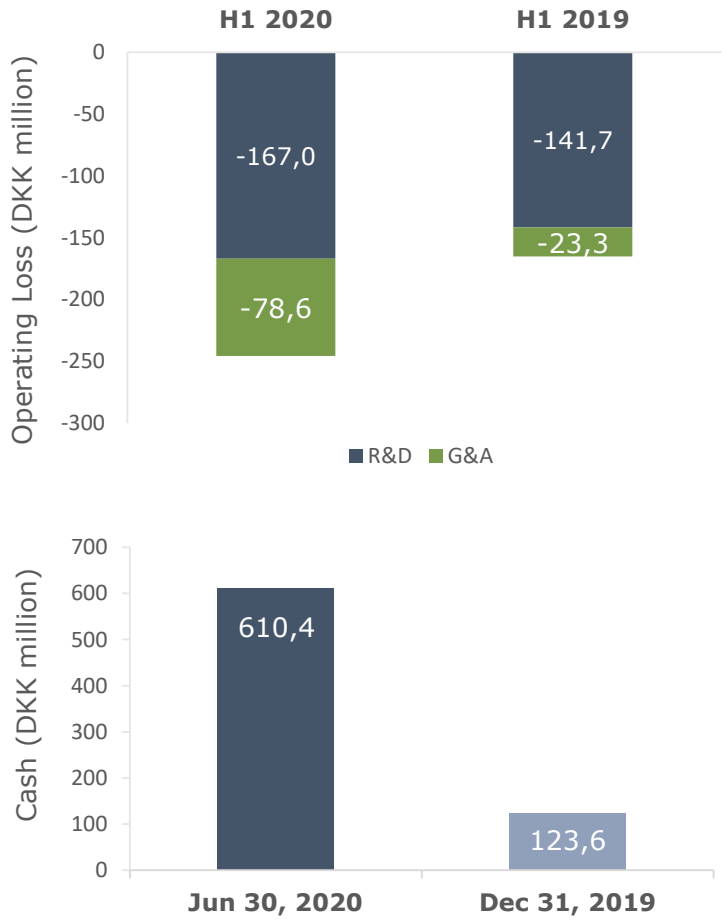
Overview H1 2020 financial results



- Operating loss increased to DKK 246 million in H1 2020 vs. DKK 165 million H1 2019
  - R&D: mainly attributed to initiation of 3 clinical pharmacology registration trials and increased number of R&D employees
  - G&A: investment in pre-launch activities including strengthening of US and CH office, medical affairs and admin expenses to support growing organization
- Net loss widened to DKK 251 million (~USD 38 million[1]) in H1 2020 vs. DKK 164 million in H1 2019
- Net Loss per share was DKK 9.88[2] (DKK 8.20 in H1 2019)
- Cash amounted to DKK 610 million (~USD 92 million) at June 30, 2020 vs. DKK 124 million as at December 31, 2019
  - Increase driven by share offering and private placement

H1 2020 represents the period 1 January 2020 to 30 June 2020. Figures are rounded. 1. Figures translated into U.S. dollars at an assumed exchange rate of DKK 6.6360 per $1.00, which was the rounded official exchange rate of such currencies as of 30 June, 2020; 2. Basic and diluted, based on 25,447,748 weighted-average shares outstanding.

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Outlook for 2020

DKK millions	2020
Operating Loss	(500) – (550)[1]
Cash position year end	>300[2]

- Increasing spend on launch preparation activities in the second half of 2020
- Costs associated with on-going clinical development activities

1. Operating loss USD (75) – (83) million; 2. cash > USD 45 million. Figures translated into U.S. dollars at an assumed exchange rate of DKK 6.6360 per $1.00, which was the rounded official exchange rate of such currencies as of 30 June, 2020.

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Concluding Remarks

Kim Stratton,
Chief Executive Officer

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Anticipated milestones over the next 12 months

† July 2020; FDA has up to 60 days to determine whether to accept the application for review; if accepted FDA has a goal to make a decision within 6 or 10 months of such acceptance time, based on whether the application is designated priority review or standard review

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Late-stage neurodegenerative orphan disease specialist potentially nearing commercialization

Investment Highlights

1. Potential near-term approval & launch of arimoclomol in NPC; NDA submission completed July 2020; submit to EMA H2 2020
2. Two registrational trial readouts expected in H1 2021 in ALS and sIBM
3. Building a highly specialized commercial footprint in US & EU
4. Underpinned by deep scientific expertise in heat-shock proteins and lysosomal biology
5. Strong financial position further improved by DKK 745 million (~$110M) offering in Feb. 2020; diversified shareholder base

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Q&A

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