



## Business Update & H1 Financial Results

August 31, 2021



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# H1 2021 Business Update

Christophe Bourdon, CEO

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# Orphazyme Today

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## WHAT WE DO

Develop drugs for rare diseases

- Global company bringing innovative drugs to patients in need
- Publicly traded in the US and Copenhagen

## WHAT WE'RE DEVELOPING

Arimoclomol (MIPLYFFA™) for NPC

- Clinically-meaningful effect on disease progression
- Durable response and good safety profile up to 36 months
- Results published in peer-reviewed Journal of Inherited Metabolic Disease
- >100 patients on active therapy in EAP

## HOW WE'LL DELIVER

Clear and focused strategy

- Obtain CHMP opinion in Q4 2021
- Conduct Type A Meeting with US FDA

# H1 Business Update

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- Announced top-line results for arimoclomol in IBM and ALS late-stage trials
- Received Complete Response Letter from FDA for arimoclomol in NPC
- Announced corporate restructuring
- Maintained ongoing dialogue with EMA on marketing authorization application for NPC
- Presented 12-month and 24-month results from OLE of arimoclomol in NPC at the Parseghian Scientific Conference
- Ended half year with DKK 334.2 million in cash



# Financial Results & Outlook

Anders Vadsholt, CFO

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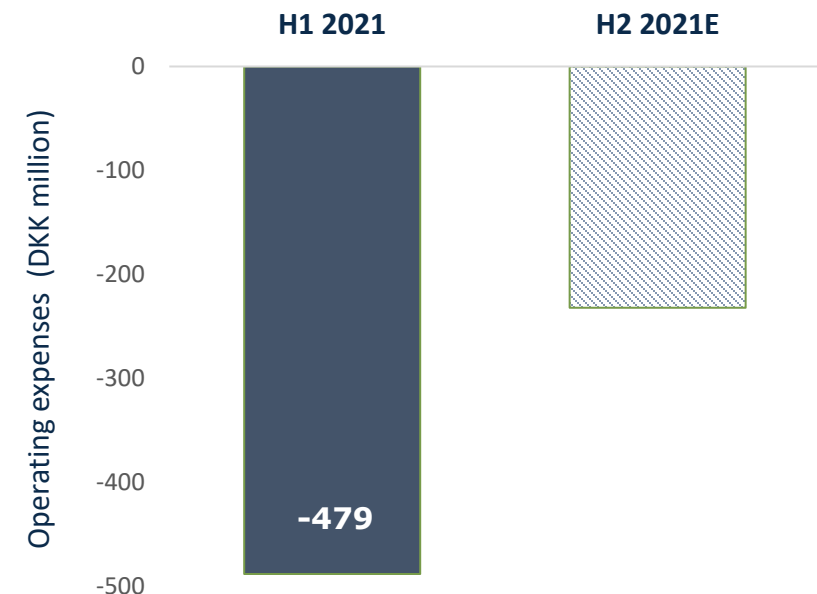


# Financial Update on Corporate Restructuring

## Core actions taken in H1'21

- Ceased development in ALS/IBM; took license impairment
- Approx. two-thirds reduction in global workforce; certain related cost savings will be recognized in H2 2021
- Core team retained to support essential activities and all medical, regulatory, and pre-commercial activities are funded

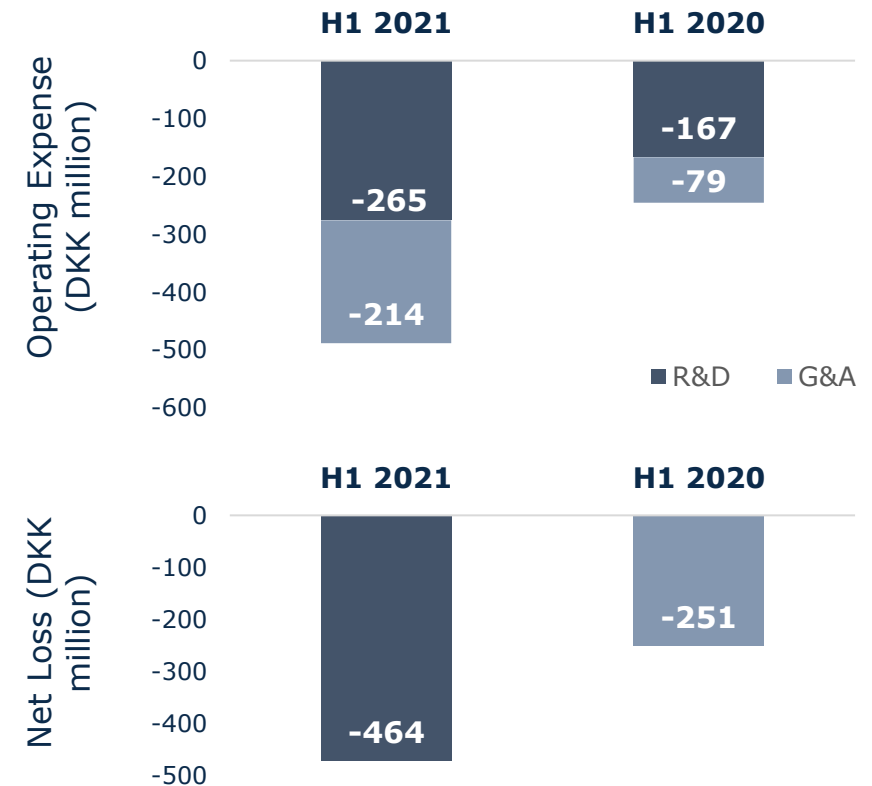
## Significant OpEx reduction in H2'21 while enabling core capabilities



# H1 Financial Results

- Net revenue of DKK 13.2M (DKK 0M, H1 2020)
  - Arimoclomol sales in France (remunerated ATU)
- Operating expense totaled DKK 479M
  - H1 2021 R&D and G&A expenses also includes certain restructuring costs and an impairment expense
- Cash as of June 30, 2021 was DKK 334.2 M vs. DKK 727M, December 31, 2020

## OpEX and net loss comparison





## Outlook & Guidance

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Confirming 2021 Financial Outlook for Operating Expense, Operating Loss and Cash Position

### **Operating Expense**

DKK 700-720M (USD 112-115M<sup>‡</sup>)

### **Operating Loss**

DKK 670-700M (USD 107-112M<sup>‡</sup>)

### **Cash Position**

~ DKK 50M at year-end (USD 8M<sup>‡</sup>)

We anticipate a net revenue of DKK 30-40M in sales related to EAP in France<sup>1</sup> (~4.8-6.4M USD<sup>‡</sup>).

1. Net revenue pertaining to ATU in France; <sup>‡</sup> Based on FX of 1 DKK per 0.16 USD.

# Closing Remarks

Christophe Bourdon, CEO

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# Clear and Focused Strategy to Deliver on Milestones

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- Advance arimoclomol to approval in both the EU and US
  - Run an efficient, global organization retaining key positions
  - Secure additional funding to support commercialization
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## H2 2021

- Expected CHMP opinion in EU
- Anticipated Type A meeting with US FDA

## H1 2022

- Expected EMA and UK MHRA approval decisions and subsequent potential commercialization in EU
- Expected resubmission of NDA for NPC to US FDA

# Questions and Answers

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