

**Company announcement**

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Inside information

**Orphazyme A/S**

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Company Registration No. 32266355

## Orphazyme appoints Christophe Bourdon as Chief Executive Officer

**Copenhagen, Denmark, March 1, 2021** – Orphazyme A/S (ORPHA.CO; ORPH) (“Orphazyme”), a late-stage biopharmaceutical company pioneering the Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, today announced the appointment of Christophe Bourdon as the company’s Chief Executive Officer, effective as of April 1, 2021, following approval from the Board of Directors.

Christophe Bourdon has a strong track record in launching and commercializing rare and non-rare disease products in Europe and the United States. He has a deep knowledge of patient journey, market access, and payer dynamics on both sides of the Atlantic. During his 25 years in the biotech/pharmaceutical industry working in three continents and holding several leadership positions, Christophe initiated and accelerated significant strategic transformations and has proven experience building successful multi-cultural and cross-functional teams. He is adept at fostering an environment focused on innovation with one constant driver in mind: Making a meaningful difference for patients. He successfully launched a variety of products in demanding environments, making him an ideal candidate to lead Orphazyme as it prepares for a potential commercial launch of its investigational product candidate, arimoclomol, in the United States and Europe.

Christophe Bourdon comes to Orphazyme from his position with Amgen, Inc. as Senior Vice President, General Manager, U.S. Oncology Business, where he led commercialization planning and execution for several products. Prior to Amgen, Christophe was Senior Vice President of Europe, Middle East, Africa, and Canada at Alexion as the company launched two breakthrough ultra-orphan drugs and negotiated payor access across UK, Germany, France, Italy, and Canada. Christophe Bourdon holds an MBA from IMD business school (Switzerland) and a BA from ISG (France).

Chairman of the Board of Directors, Georges Gemayel, states *“I am delighted to announce Christophe Bourdon will join our leadership team as Chief Executive Officer starting April 1, 2021. This is an important time for Orphazyme, with numerous near-term milestones that will shape the company’s future direction. Our Board conducted an extensive search for a leader who can guide not only our near-term execution, but also align the company around a vision for impact and scale for our long-term growth ambitions. With his strong track record on growth over two decades in relevant roles, Mr. Bourdon will bring his dynamic leadership and skillset to successfully build out and integrate our organization as we prepare for the anticipated commercial launch of our investigational product candidate, arimoclomol, in the United States and Europe. As an authentic and result-oriented leader, he believes in building a transparent and inclusive corporate culture and is the right choice to lead Orphazyme into its next growth phase.”*

Christophe Bourdon states *“The opportunity to join Orphazyme was compelling for me, not only based on its purposeful mission, but also the incredible near-term opportunities to create impact for patients. It is both exciting and humbling to assume this role at such a pivotal time,”* said Mr. Bourdon. *“Building on the learnings I have gained from my invaluable experience at Amgen and the rare disease experience garnered at Alexion, I look forward to championing this talented team to advance the mission on behalf of our patient communities and deliver value for our shareholders. I am also very much looking forward to relocating to Copenhagen.”*

**For additional information, please contact****Orphazyme A/S**

Anders Vadsholt, Interim CEO and CFO

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**About Orphazyme A/S**

Orphazyme is a late-stage biopharmaceutical company pioneering the Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases. The company is harnessing amplification of Heat-Shock Proteins (or HSPs) in order to develop and commercialize novel therapeutics for diseases caused by protein misfolding, protein aggregation, and lysosomal dysfunction, including lysosomal storage diseases and neuromuscular degenerative diseases. Arimoclomol, the company's lead candidate, is in clinical development for four orphan diseases: Niemann-Pick disease Type C (NPC), Amyotrophic Lateral Sclerosis (ALS), Inclusion Body Myositis (IBM) and Gaucher disease. Orphazyme is headquartered in Denmark and has operations in the U.S. and Switzerland. Orphazyme's shares are listed on Nasdaq U.S. (ORPH) and Nasdaq Copenhagen (ORPHA).

**About arimoclomol**

Arimoclomol is an investigational drug candidate that amplifies the production of Heat-Shock Proteins (HSPs). HSPs can rescue defective misfolded proteins, clear protein aggregates, and improve the function of lysosomes. Arimoclomol is administered orally and has now been studied in seven phase 1, four phase 2 and one pivotal phase 2/3 trial. Arimoclomol is in clinical development for NPC, Gaucher Disease, sIBM, and ALS. Arimoclomol has received orphan drug designation (ODD) for NPC, IBM, and ALS in the US and EU. Arimoclomol has received fast-track designation (FTD) from the U.S. Food and Drug Administration (FDA) for NPC, IBM and ALS. In addition, arimoclomol has received breakthrough therapy designation (BTD) and rare-pediatric disease designation (RPDD) from the FDA for NPC.

**About NPC**

Niemann-Pick disease Type C (NPC) is a rare, genetic, progressively debilitating, and often fatal neurovisceral disease. It belongs to a family known as lysosomal storage diseases and is caused by mutations leading to defective NPC protein. As a consequence, lipids that are normally cleared by the lysosome accumulate in tissues and organs, including the brain, and drive the disease pathology. We estimate the incidence of NPC to be one in 100,000 live births and the number of NPC patients in the United States and in Europe to be approximately 1,800 individuals. There are no approved treatments for NPC in the U.S.

**Forward-looking statement**

This company announcement 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.