



## Hansa Biopharma and Sarepta Therapeutics agreement

*Lund, Sweden  
July 2<sup>nd</sup> , 2020*



*...at Hansa Biopharma we envision a world where all patients with  
rare immunologic diseases can lead long and healthy lives...*

# Forward-looking statement

This presentation may contain certain forward-looking statements and forecasts based on uncertainty, since they relate to events and depend on circumstances that will occur in the future and which, by their nature, will have an impact on Hansa Biopharma's business, financial condition and results of operations. The terms "anticipates", "assumes", "believes", "can", "could", "estimates", "expects", "forecasts", "intends", "may", "might", "plans", "should", "projects", "will", "would" or, in each case, their negative, or other variations or comparable terminology are used to identify forward-looking statement. There are a number of factors that could cause actual results and developments to differ materially from those expressed or implied in a forward-looking statement or affect the extent to which a particular projection is realized. Factors that could cause these differences include, but are not limited to, implementation of Hansa Biopharma's strategy and its ability to further grow, risks associated with the development and/or approval of Hansa Biopharma's products candidates, ongoing clinical trials and expected trial results, the ability to commercialize imlifidase, technology changes and new products in Hansa Biopharma's potential market and industry, the ability to develop new products and enhance existing products, the impact of competition, changes in general economy and industry conditions and legislative, regulatory and political factors.

No assurance can be given that such expectations will prove to have been correct. Hansa Biopharma disclaims any obligation to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

# Hansa Biopharma and Sarepta Therapeutics Inc. enter into exclusive agreement in DMD and LGMD gene therapy

## Today's agenda

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- 1) Scope of the Hansa-Sarepta agreement
  - 2) Issues with neutralizing antibodies in gene therapy
  - 3) Highlights from recent article in Nature Medicine
  - 4) The structure of the collaboration and financials
  - 5) Unlocking the gene therapy opportunity for NAb positive patients in Duchenne and Limb-girdle diseases
  - 6) Q&A
- Appendix



# The scope of our partnership agreement with Sarepta

First partnership agreement in gene therapy for Hansa Biopharma



- Partnership agreement underpins the unique features of imlifidase as a potential pre-treatment to NAb positive patients in gene therapy

Sarepta obtains a global and exclusive license to imlifidase in two indications



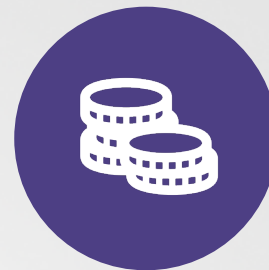
- Sarepta obtains a global and exclusive license to develop and promote imlifidase as pre-treatment in patients with Duchenne muscular dystrophy (DMD) and Limb-girdle muscular dystrophy (LGMD)

Sarepta to absorb development costs associated with imlifidase in gene therapy\*



- Sarepta will be responsible for conducting pre-clinical and clinical studies and subsequently applying for regulatory approval in DMD and LGMD indications
- Sarepta will also be responsible for promotion of imlifidase as pre-treatment to gene therapy following potential approval

Very significant economics and value creation opportunity



- Upfront payment of USD 10m
- Up to USD 397,5m in potential milestone payments
- Royalties paid to Hansa on gene therapy sales enabled through pre-treatment with imlifidase
- Incremental imlifidase sales

\* In select indications: Duchenne muscular dystrophy (DMD) and Limb-girdle muscular dystrophy (LGMD)

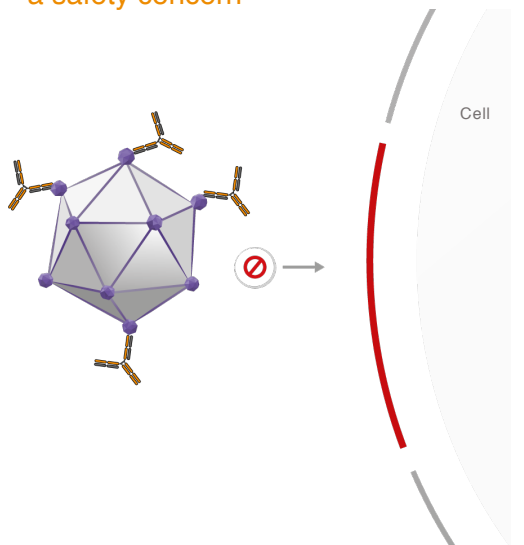


# Neutralizing antibodies (Nabs) are immunological barriers in gene therapy

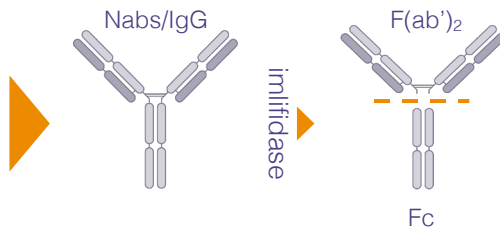
*Between approximately 5% and 70%<sup>1,2</sup> of patients considered for gene therapy treatment carry neutralizing anti-AAV antibodies forming a barrier for treatment eligibility*

*Our hypothesis is that imlifidase has the potential to eliminate neutralizing antibodies as a pre-treatment, prior to the introduction of gene therapy*

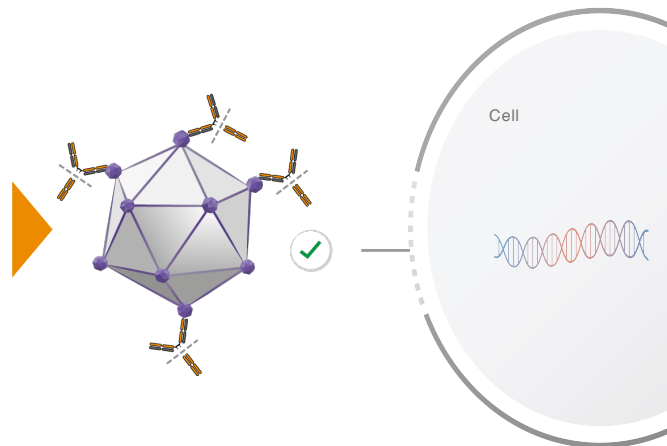
- 1 Antibodies prevent effective transfer of healthy gene sequence and can be a safety concern



- 2 Imlifidase is a unique IgG antibody-cleaving enzyme that cleaves IgG at the hinge region with extremely high specificity



- 3 The idea is to eliminate the neutralizing antibodies as a pre-treatment to enable gene therapy



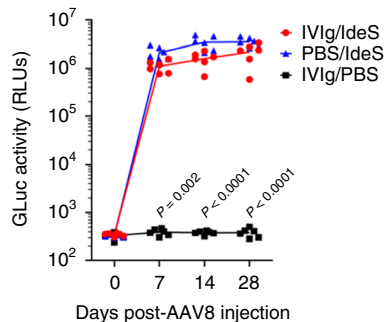
# Imlifidase (IdeS) was highlighted in Nature Medicine<sup>1</sup> with encouraging outcome

## Results from preclinical studies with imlifidase (ideS) in gene therapy demonstrate imlifidase as a potential solution to overcome pre-existing antibodies to AAV-based gene therapy



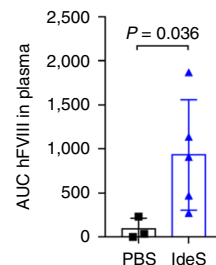
### Imlifidase tested in a hemophilia mouse model

- Imlifidase decreased anti-AAV antibodies and enabled efficient gene transfer



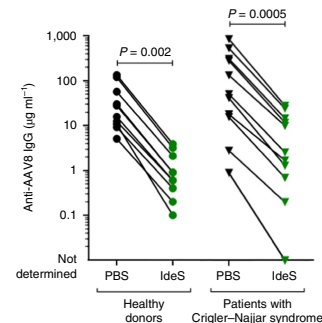
### Imlifidase tested in NHP ahead of AAV vector infusion

- Pre-treatment with imlifidase in anti-AAV positive nonhuman primates (NHP) ahead of AAV vector infusion was safe and resulted in enhanced liver transduction and hFVIII plasma levels



### Imlifidase tested in human plasma samples (GT patients)

- Imlifidase reduced anti-AAV antibody levels from human plasma samples in vitro, incl. plasma from prospective gene therapy trial participants



<sup>1</sup> Nature Medicine <https://doi.org/10.1038/s41591-020-0911-7>

Leborgne et al. Nat Med (2020)

# We aim to unlock the potential for our enzyme technology in gene therapy by leveraging the scientific approaches and leadership in the space

## A unique opportunity to combine efforts...

...and to use the unique features of imlifidase to potentially enable gene therapy treatment in patients who today aren't eligible for these breakthrough therapies due to pre-existing neutralizing antibodies in two indications with a very high unmet medical need

## Structure of the partnership

Sarepta will be responsible for conducting

- Pre-clinical/clinical studies with imlifidase
- Regulatory approvals
- Promotion of imlifidase as a pre-treatment to Sarepta's gene therapies following potential approval

Hansa will supply product, support with know-how and involve in the regulatory approval process

## Hansa's financial participation

Potential total deal value for Hansa amounts to up to USD ~400m plus royalties and incremental imlifidase sales



### Hansa's key competences

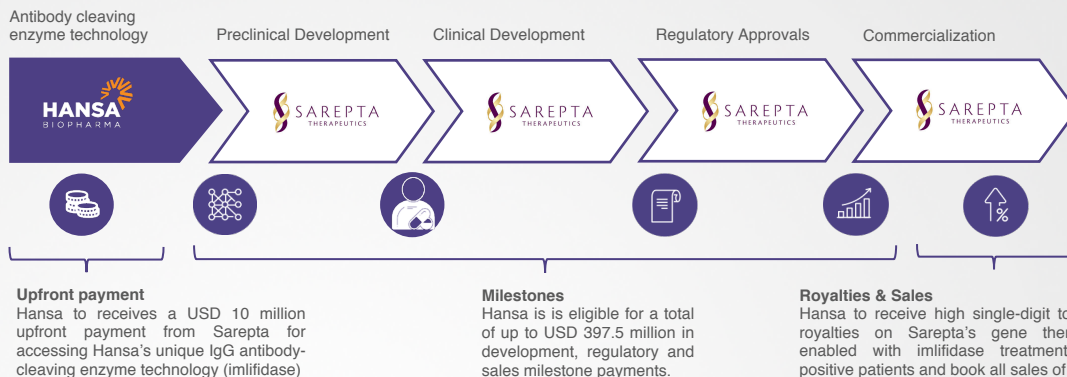
- Leader in immunomodulatory enzyme technology for rare IgG mediated diseases
- Strong experience in antibody cleaving and desensitization
- Broad enzyme technology that can be used in a variety of indications





### Sarepta's key competences

- Market leader within gene therapy targeted at muscular dystrophies
- Strong pre-clinical and clinical gene therapy portfolio
- Scientific approach and knowledge within gene therapy
- Experience with challenges of Nab-positive patients

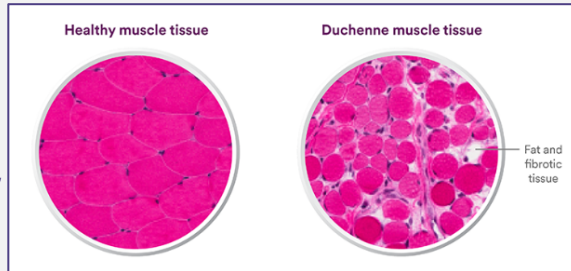


# Sarepta obtains a global and exclusive license to imlifidase in DMD and LGMD in gene therapy

## About Duchenne muscular dystrophy (DMD)

- Duchenne muscular dystrophy is a rare genetic disease caused by mutation in the DMD gene, encoding for the protein dystrophin
- Muscles in the body become weak and most patients use wheelchair by the age of 12
- Affects one in 3,500 to 5,000 males born worldwide (approximately 400-500 annual male cases in the US) of which approximately 15-20% are estimated to have pre-existing antibodies to AAV-based gene therapy which prevents the patients from being treated with gene therapy

*"On average, every day DMD takes the life of a child in the United States..."*

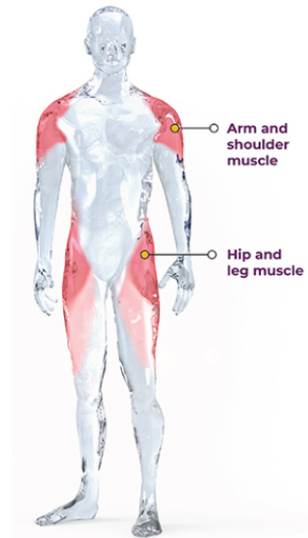


Source: Sarepta Therapeutics

<https://investorrelations.sarepta.com/static-files/0c4aca61-9419-45a5-afb1-ff2092644627>

## About Limb-girdle muscular dystrophy (LGMD)

- Limb-girdle muscular dystrophy is a group of diseases that cause weakness and wasting of the muscles
- May be caused by a single gene defect affecting specific proteins within muscle cells
- Global prevalence of 1.63 per 100,000 individuals (of which approximately 15-20% are estimated to have pre-existing antibodies to AAV-based gene therapy which prevents patients from being treated with gene therapy)



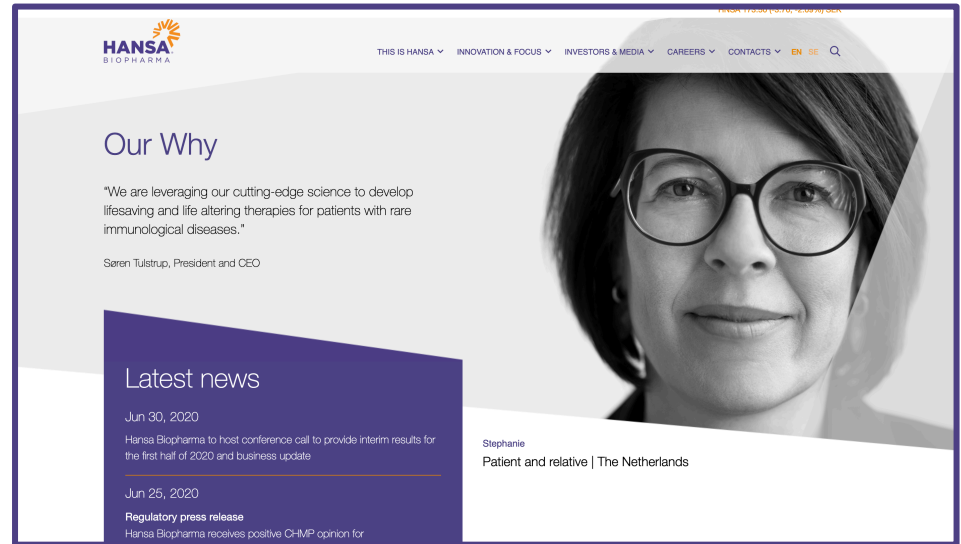
Source: Sarepta Therapeutics

<https://investorrelations.sarepta.com/static-files/0c4aca61-9419-45a5-afb1-ff2092644627>

# Q&A

*... at Hansa Biopharma we envision a world  
where all patients with rare immunologic  
diseases can lead long and healthy lives...*

Visit our web site  
[www.hansabiopharma.com](http://www.hansabiopharma.com)



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## Calendar

Jul 16, 2020

Interim Report Jan-Jun 2020

Aug 14, 2020

Nordea Small & Mid Cap Seminar, Stockholm (virtual)

Sep 3, 2020

Pareto Healthcare Conference, Stockholm (virtual)

Sep 14-15, 2020

Morgan Stanley Global Healthcare Conference, NYC (virtual)

Sep 16, 2020

BofAML Global Healthcare Conference, London (virtual)

Sep 23, 2020

ABG Small & Mid Cap Seminar, Copenhagen

Oct 22, 2020

Interim Report Jan-Sep 2020

Nov 25, 2020

Ökonomisk Ugebrev Life Science Conference, Copenhagen



# Appendix



# Hansa Biopharma at a glance



## Company background

- Founded 2007 with HQ in Lund, Sweden
- Søren Tøstrup, CEO – Ulf Wiinberg, Chairman
- ~80 employees (~2/3 in R&D) June, 2020
- Operations in Sweden, US & across Europe
- Market cap: SEK ~7bn (USD ~700m) June 2020
- Listed on Nasdaq OMX Stockholm (HNSA)



## Leader in immunomodulatory enzymes for rare IgG-mediated diseases

- Imlifidase is a unique IgG antibody-cleaving enzyme. If approved, Imlifidase may have the potential to meet a large unmet need and preserve and transform the lives of people with rare diseases
- Imlifidase has been studied in five clinical studies in kidney transplantation
- Imlifidase has been published in peer-reviewed journals (e.g. New England Journal of Medicine and the American Journal of Transplantation)



## Broad pipeline in transplantation and autoimmune diseases

- Lead indication in kidney transplantation in highly sensitized patients
  - EU: positive CHMP opinion received on June 2020, EU approval expected Q3 2020
  - US: Study protocol submitted June 2020, study expected to be initiated Q4 2020
- Anti-GBM antibody disease (Phase 2)
- Antibody mediated kidney transplant rejection (AMR) (Phase 2)
- Guillain-Barré syndrome (GBS) (Phase 2)
- NiceR - Recurring treatment in autoimmune disease, transplantation and oncology (Preclinical)
- EnzE – Cancer immunotherapy (Preclinical)



## Key financials

|                          |                  |                 |
|--------------------------|------------------|-----------------|
| • Cash position          | Q1'20* SEK 477m  | FY'19 SEK 601m  |
| • R&D expenses           | Q1'20* SEK -53m  | FY'19 SEK -193m |
| • Operating Profits/Loss | Q1'20* SEK -91m  | FY'19 SEK -360m |
| • Operating cash flow    | Q1'20* SEK -121m | FY'19 SEK -335m |

\* Unaudited financials

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# Hansa Biopharma and Sarepta Therapeutics enters a partnership in gene therapy

## About Sarepta Therapeutics Ltd.

- US-based biopharma developing precision genetic medicines to treat rare neuromuscular and central nervous system diseases
  - Headquartered in Cambridge, Massachusetts, USA
  - More than 700 employees
  - Revenue of ~400 USDm 2019
  - 12,5 bn USD market cap
- World leader in development of gene therapy for muscular dystrophies including Duchenne muscular dystrophy (DMD) and Limb-girdle muscular dystrophy (LGMD)



# Potential indication universe

