HANSA BIOPHARMA

Corporate Presentation



Forward-looking statements

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The factors set forth above are not exhaustive and additional factors could adversely affect our business and financial performance. We operate in a very competitive and rapidly changing environment, and it is not possible to predict all factors, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements. Given these risks and uncertainties, investors should not place undue reliance on forward-looking statements as a prediction of actual results.

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Table of Contents



| Company Overview strategy, science, pipeline | slides 3 – 11 |
|--|----------------|
| Therapy Areas autoimmune, gene therapy, transplantation | slides 12 - 26 |
| Executive Summary | slide 28 |
| Additional Resources ESG, patents, ownership, leadership, references | slides 29 - 36 |
| | |

Hansa Biopharma – a commercial stage biotech delivering new medicines for rare immunological diseases



SCIENTIFIC INNOVATION

- Proprietary immunomodulating technology platform
- Seven clinical trials in autoimmune, gene therapy, transplantation
- Expansive indication universe in rare / ultra rare disease



COMMERCIAL STAGE

- First and only treatment approved for desensitization in kidney transplantation
- Utilization in major EU markets and
- US BLA submission in 2025



LONG-TERM POTENTIAL

- Backed by leading specialist investors - financing into 2026
- Strong IP protections through 2041
- Diverse, engaged culture with 30+ different nationalities
- HQ in Lund, Sweden







- Broaden imlifidase label beyond kidney transplantation
- Develop next generation IgG enzyme for redosing
- Expand technology platform and pipeline into autoimmune & gene therapy



- Successfully launch IDEFIRIX (imlifidase) in Europe
- Secure US approval and launch
- Seek partnerships to accelerate growth & reduce risk



- Build a focused, agile and empowered global organization
- Expand geographically
- Deliver priorities within the context of Sustainability

Imlifidase is an innovative, first in class molecule with a proven approach to inactivate IgG





IgG cleaving enzymes uniquely positioned through rapid onset of effect and therapeutic reach



Comparison of established therapies to significantly reduce IgG levels within a few weeks

PLEX = plasma exchange

IgG cleaving enzymes provide unmatched speed of antibody decrease gG % vs baseline Conceptual illustration \cap 2 10 5 8 Week



IgG cleaving enzymes cleave antibodies

across all domains

The speed and reach of IgG cleavage puts Hansa's enzymes in a unique position

IgG levels in FcRn inhibition and PLEX depend on the frequency of treatment

B cell depletion potentially has a faster and larger effect on autoantibodies, as compared to total IgG

HNSA-5487 is a next generation molecule that can very rapidly and robustly reduce IgG with clear redosing potential



HNSA-5487

- Next generation IgG cleaving enzyme based on an animal pathogen
- Inactivates IgG by cleaving the heavy chains of IgG, effectively eliminating the Fc-dependent effector functions
- Engineered for high potency, specificity and safety
- Immunogenicity profile that supports redosing potential
- Safe and well tolerated

Rapid & robust **IgG** reduction

Address

R

- Reduces IgG levels by more than 95 percent within a few ٠ hours
 - IgG levels returning to normal range six months after initial dosing
- As efficacious as imlifidase in reducing total IgG levels ٠

| Redosina | • | Lower ADA pre-treatment levels and significantly reduced |
|-----------|---|--|
| notontial | | ADA response as compared to imlifidase |
| potential | • | Efficient IgG reduction in serum samples at six- and 12- |

Focus where autoantibodies drive the disease

months post initial dose

- Need for management of symptoms at onset of disease unmet need and during recurrent immune system attacks
 - Diseases with little to no advanced treatment options

Hansa's proprietary technology platform with two unique molecules and broad potential indications





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Broad clinical pipeline in autoimmune, gene therapy and transplantation



| | Preclinical | Phase 1 | Phase 2 | Phase 3 | Marketing authorization | Marketed | Partner | Status | Next anticipated milestone |
|--|-------------|---------|---------|---------|-------------------------|----------|----------|--|--|
| Imlifidase | | | | | | | | | |
| EU: Kidney transplantation in highly sensitized patients ^{1,2} | | | | • | | | | Commercialization ongoing Post approval Clinical Phase 3 ongoing | EU: Additional agreements around reimbursement / Post authorization study to be completed by end of 2025 |
| U.S. "ConfldeS": Kidney transplantation in highly sensitized patients ^{1,2} | | | | | | | | Clinical Phase 3 ongoing | Data readout in 2H 2025 |
| GOOD-IDE S-02: Anti-GBM antibody disease | | | | | | | | Clinical Phase 3 ongoing | Complete enrolment (50 patients) |
| 16-HMedIdes-12: Active Antibody Mediated Rejection (AMR) | | | | | | | | Clinical Phase 2 completed | Publication in peer-reviewed journal |
| 15-HMedIdeS-09: Guillain-Barré Syndrome (GBS) | | | | | | | | Clinical Phase 2 ongoing | Comparative efficacy analysis 2024 |
| Investigator-initiated trial in ANCA- associated vasculitis ³ | | | | | | | | Clinical Phase 2 ongoing | Complete enrolment (10 patients) |
| SRP-9001-104: Pre-treatment ahead of gene therapy in Duchenne Muscular Dystrophy (DMD) | | | | | | | S AREPTA | Clinical Phase 1b ongoing | Complete en rolment |
| Pre-treatment ahead of gene therapy in Limb-Girdle Muscular Dystrophy (LGMD) | | | | | | | SAREPTA | Preclinical research ongoing | Preclinical research |
| Pre-treatment ahead of gene therapy in Pompe disease | | | | | | | 🛞 AskBio | Preclinical research ongoing | Preclinical research |
| Pre-treatment a head of gene therapy in Crigler-Najjar syndrome | | | | | | | | Preclinical research ongoing | Commence clinical study |
| HNSA-5487 | | | | | | | | | |
| NICE-01: HNSA-5487 – Lead candidate from the NiceR program | | | | | | | | Clinical Phase 1 completed | Alignment with regulatory authorities on development path |

1 Results from the Phase 1 study have been published, Winstedt et al. (2015) PLOS ONE 10(7)

² Lorant et al., American Journal of Transplantation and 03+04 studies (Jordan et al., New England Journal of Medicine) ³ Investigator-initiated study by Dr. Adrian Schreiber and Dr. Philipp Enghard, at Charité Universitätsmedizin, Berlin, Germany



Advancing the science in autoimmune, gene therapy, and transplantation

Delivering the Pipeline

- 7 Ongoing clinical trials in autoimmune, gene therapy and transplantation
- 1 Additional gene therapy study to commence in 2024

Advancing the Science

Publications in peer-reviewed journal

8 Presentations at leading medical congresses







Nephrologie 2024 IMLIFIDASE IgG cleaving therapy with a unique MOA

AUTOIMMUNE

15-HMedideS-09 Ph 2 (GBS) *Trial Complete Addt'I Data Readout Q4 2024*

GOOD-IDES-02 Ph 3 (anti-GBM disease) Ongoing Data Readout 2H 2025

GENE THERAPY SRP-9001-104 Ph 1b (DMD) Trial Commenced Data Readout 2025

Genethon Preclinical (Crigler-Najjar) Trial to Commence Q4 2024

AskBio Preclinical (Pompe disease) Trial to Commence 2025

HNSA-5487 next-gen IgG cleaving molecule with redosing potential

NICE-01 Ph 1 Trial Complete Additional Analysis Complete Clinical Development Path Annc

TRANSPLANTATION

US ConfldeS Ph 3 (kidney) Enrolment Completed Data Readout 2H 2025

16-HMedideS-12 Ph 2 (AMR) *Trial Complete Data published July 2024*

Post Authorization Efficacy Ph 3 (kidney) Ongoing Data readout 2025



Table of Contents



Company Overview strategy, science, pipeline

slides 3 - 12

Therapy Areasslides 13 - 27autoimmune, gene therapy, transplantation

Executive Summary

slide 28

Additional Resources ESG, patents, ownership, leadership slides

Autoimmune



Imlifidase may have relevance in several autoimmune diseases where IgG plays an important role in the pathogenesis



In Phase 2 trials, imlifidase was shown to cleave autoantibodies and stop disease progression in monophasic conditions

PROOF OF CONCEPT IN TWO INDICATIONS

Anti-GBM disease

Imlifidase Ph 2 results

67% of patients were dialysis

independent after six months vs. the

historical cohort, where only 18% had functioning kidney

Phase 3 ongoing

70% enrolled

1.6 in a million affected annually

Guillain-Barré Syndrome

1.7-4.3 per 100,000 annually

Imlifidase Ph 2 results

Rapid improvement across several efficacy outcome measures, as compared with previously published data

Phase 3 plans under review



HNSA-5487 may enable expansion into acute flares in chronic conditions through redosing

Gene Therapy

GENE THERAPY



Imlifidase may enable gene therapy treatment in rare disease patients with pre-existing antibodies

AAVs

the delivery system of gene therapy²⁶⁻²⁹

5-70%

patients considered for gene therapy have anti-AAV antibodies²⁶

Pre-existing antibodies

excludes patients from trials & treatment³⁰

Eliminate antibodies as a pretreatment to enable gene therapy



Partnership strategy

Global partnerships with gene therapy companies



- World leader within gene therapy targeted at muscular dystrophies
- High level read out in DMD expected 2025



- Early innovator in gene therapy
- Encouraging preclinical data presented at ASGCT 2024



- > A not-for-profit pioneer in gene therapies
- Preclinical work underway in Crigler Najjar syndrome



Systemic gene therapy is an emerging opportunity



Global exclusive agreements with leading gene therapy companies in select indications





CAPABILITIES & RESOURCES

- Early innovator in gene therapy
- Conducts pre-clinical and clinical trials (Phase 1/2)



CAPABILITIES & RESOURCES

- A pioneer in the discovery and development of gene therapies
- Conducts pre-clinical and clinical trials (Phase 1/2)



CAPABILITIES & RESOURCES

- World leader in gene therapy in muscular dystrophies
- Pre-clinical and clinical plan
- Regulatory & Promotion
- FDA approval in 2023

INDICATION EXCLUSIVITY

Pompe Disease - ~ 5,000 – 10,000 patients in the US and EU. In addition, 1 in 40,000 births (200 cases)

In addition, 1 in 40,000 births (200 cases) are diagnosed yearly.

INDICATION EXCLUSIVITY

Crigler-Najjar syndrome - approximate incidence is 0.6-1 case per one million people or 600 patients in Europe and the U.S

INDICATION EXCLUSIVITY

Duchenne Muscular Dystrophy (DMD) -1/3,500 to 5,000 male births worldwide Limb-Girdle Muscular Dystrophy - global prevalence of ~1.6 per 100k individual Imlifidase pre-treatment decreases pre-existing antibodies and enhances transduction and transgene expression in animal models





Data from a nimal models

*P<0.05. †Data are represented as mean ± SEM and analyzed by one-way ANOVA followed by post-hoc analysis with Dunnett's multiple comparison test . ‡Data are represented as the mean ± SEM for the percent area for all of the muscle tissues analyzed at terminal necropsy. \$AAVrh74 titer ≤1:400. \$AAVrh74 titer 1:800–1:1600.

AAV, adeno-associated virus; AAVrh74, adeno-associated virus; hesus isolate serotype 74; Ab, antibody, a.u., arbitrary units; eGFP, enhanced green fluorescent protein; NHP, non-human primate; ns, not significant; vg, viral genome.

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Transplantation



Imlifidase may enable incompatible kidney transplantation in highly sensitized patients 170k*

patients waiting for a kidney transplant⁵⁰

DSAs

Donor specific antibodies that reject organ transplantation SOC

based on organ compatibility **10-15%**

patients with DSAs – or highly sensitized - unlikely to be transplanted due to incompatibility⁵¹

Long term data from 17-HMedIdeS-14 study in kidney transplantation confirms sustained benefit of imlifidase^{52,53}



- > 82% five-year graft survival
- > 90% patient survival rate
- > 50 ml/min/m2 eGFR

Conditional EU approval for desensitization in kidney transplant patients⁵⁴



Addresses the limitations of other modalities



The **first and only** approved drug to enable incompatible kidney transplants⁵⁰



Market access in 75% of the EU transplant market

Two key studies for imlifidase



17-HMedIdeS-14

Long-term follow-up study

Extended pooled analysis from the 17-HMedIdeS-14 study

A long-term follow-up study of patients who have received a kidney transplant following desensitization with imlifidase.

RESULTS

- 82% graft survival
- 90% patient survival rate
- 50 ml/min/m² eGFR

Further data from extended pool analysis expected in 2024

ConfldeS US Ph 3 pivotal trial

Open-label, controlled, randomized trial evaluating 12-month kidney function in highly sensitized kidney transplant patients with positive crossmatch against a deceased donor, comparing desensitization using imlifidase with standard of care

RESULTS

- Randomization completed (May 2024)
- 156 patients screened & enrolled
- **23** sites active and 11 sites have already dosed with imlifidase

US FDA BLA to be filed in 2H 2025

At a Glance: IDEFIRIX launch in Europe

Strategic Levers



Market Access



Clinical Readiness

Organ allocation

Patient selection and treatment

Launch Progress

IDEFIRIX was launched in 2020 as the **only desensitization strategy** for highly sensitized kidney transplant patients:

15 markets with reimbursement (75% of EU transplant market)

Country issued clinical guidelines

32

centers with experience

9.3m USD in product

sales in 2023

113

clinics IDEFIRIX ready to treat

clinical

Scaling Globally

Transforming the desensitization treatment landscape and advance a new way of transplanting patients

Build the foundation Commercialize in key markets Ensure clinical readiness Support patient/organ access

NOW (1-3 vrs)

Grow internationally Secure US approval Go beyond core markets Full EU authorization

Expand label Living donor Other solid organs

LONG-TERM

MID TERM (3-5 vrs)

Solid commercial opportunity in kidney transplantation desensitization





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Idefirix Launch in Europe

Market size

3,000 - 4,000

Commercial progress

>

25

14 countries with market access

~75% of transplant volume

~50 Idefirix "ready"

17 centers with centers with clinical experience repeat usage

European launch has reached inflection point with increasing adoption across major markets Phase 3 Nearing Completion

Market size

1,000-1,500

Phase 3 trial

Mav fully randomized Phase 3 trial

2H 2025 **BLA** filina

24 centers involved in trial

>20% of transplant volume

Broad clinical experience creates foundation for fast commercial uptake



anti-HLA

Opportunities for imlifidase in transplantation beyond kidney



Lung transplantation desensitization

10-15% of lung transplant recipients have some degree of presensitization to allo-antigens.57 De DSAs occur post transplantation in up to 55% of all lung transplant patients.58

Antibody-mediated rejection in lung transplantation



The actual incidence of luna transplantation AMR is unknown, with studies that define AMR by the ISHLT consensus definition report incidence of up to 27% of lung transplant patients.58



Antibody-mediated rejection in heart transplantation

Heart transplantation desensitization

Up to **20%** of heart transplant

AMR in 10–20% of occurs patients after heart transplant, typically, within a few months after transplant. 25% of cases occur after than one more vear transplantation.60



Table of Contents



| Company Overview strategy, science, pipeline | slides 3 – 12 |
|--|----------------|
| Therapy Areas autoimmune, gene therapy, transplantation | slides 13 - 27 |
| Executive Summary | slide 28 |
| Additional Resources ESG, patents, ownership, leadership, references | slides 30 - 36 |
| | |



Hansa Biopharma has the potential to lead the way in rare immunological diseases

Commercial stage, first-in-class asset



Commercial stage IgG cleaving enzyme with long-term data

Commercial-scale manufacturing supports launches

Over 200 patients treated

Validated pipeline



Paradigm shift for kidney transplantation

7 ongoing trials in autoimmune and gene therapy

Next gen enzyme could unlock **broader indication universe**





Publicly traded on **NASDAQ Stockholm**

Considering dual-listing in the **US**

Strong IP portfolio, with coverage until the 2040s

2024 milestones **COMPLETED** Randomization in US ConfldeS trial **COMPLETED** HNSA-5487 12 mth analysis and clinical dev pathway

Full data readout in GBS Phase 2



Table of Contents



| Company Overview strategy, science, pipeline | slides 4 – 11 |
|--|----------------|
| Therapy Areas autoimmune, gene therapy, transplantation | slides 13 - 26 |
| Executive Summary | slide 28 |
| Additional Resources ESG, patents, ownership, leadership, references | slides 30 - 36 |
| | |



Hansa Sustainability Priorities and Approach



HEALTHY PEOPLE

Address the unmet needs of people with rare diseases by developing life-saving treatments, ensuring sustainable and equitable access to care, and putting them at the center of our business



HEALTHY BUSINESS

Be an ethical, transparent business with the highest integrity and standards driving personal accountability for all. Cultivate a culture of collaboration and innovation grounded in individual development, and benefits that drive performance in a healthy, safe work environment



HEALTHY PLANET

Embrace sustainable decision making and environmental stewardship by becoming a default sustainable business from discovery and clinical trials, to product launches and manufacturing

Strong technology protection through patents and orphan drug designations



- Our lead enzyme, imlifidase, is protected by six patent families including both granted patents and pending applications and cover the use of imlifidase
- Patents cover use of imlifidase at least in:



HNSA-5487 patent coverage to at least 2041*

- Our next-generation enzyme, HNSA-5487, is protected by currently three granted/pending patent families as applicable
- Patent coverage in key markets, i.e. USA, EU, UK, JP, AU and more
- Based on a standard 20-year term, without any extensions, HNSA-5487 has patent protection until:



*Excluding potential patent term extensions (PTE) and supplementary protection certificates (SPC) and data exclusivity 'Idefrix is indicated for desensitisation treatment of highly sensitised adult kidney transplant patients with positive or oss match against an available deceased donor. The use of Idefrix should be reserved for patients unlikely to be transplanted under the available kidney allocation system including prioritisation programmes for highly sensitised patients.





Ownership in Hansa Biopharma

Top 10 shareholders as per September 30, 2024*

| Shareholder Name | No. of Shares | Ownership % |
|--------------------------|---------------|-------------|
| Redmile Group LLC | 13,156,700 | 19.40% |
| Braidwell LP | 8,247,600 | 12.16% |
| Theodor Jeansson Jr. | 2,720,000 | 4.01% |
| Hansa Biopharma AB | 2,204,667 | 3.25% |
| Nexttobe AB | 2,155,379 | 3.18% |
| Fjärde AP-fonden | 2,094,000 | 3.09% |
| Thomas Olausson | 1,917,000 | 2.83% |
| Avanza Pension | 1,906,416 | 2.81% |
| Handelsbanken Fonder | 1,724,561 | 2.54% |
| Sphera Funds Management | 1,107,000 | 1.63% |
| Other | 30,580,918 | 45.10% |
| Total shares outstanding | 67,814,241 | 100.00% |

Classification of ownership as per January 31, 2024



* Following execution of a directed share issue of SEK 372m (USD 34.6m) on April 12, 2024, the number of outstanding shares increased to 67,814,241 shares.

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Experienced Board and Executive Committee



Extensive experience from the global healthcare industry

Executive Committee



Søren Tulstrup President & CEO (2018) +30 years in the Healthcare sector

Evan Ballantyne

+30 years in the life science sector

Former CEO at Gain Therapeutics.

OncXerna Therapeutics, and Orchestra

SVP & CFO (2024)

Shareholding: 0

BioMed

Former CEO at Vifor Pharma Former SVP at Shire Pharmaceuticals Former CEO at Santaris Pharma Shareholding: 50,347

Hitto Kaufmann Chief R&D Officer (2023) +20 years in R&D Former CSO at Pieris Pharmaceuticals For mer Head of Strategy and Operations at Sanofi

Anne Säfström Lanner

Former Head of HR Cellavision

Former Head of HR European Spallation Source

SVP & CHRO (2019)

Shareholding: 7,273

Shareholding: 0

Board of Directors



Peter Nicklin Chairman (2022)

Versantis

+30 years in the Healthcare sector Chairman of Tunstall Healthcare, Sciensus &

Held senior executive roles at Baxter, Bayer, Novartis & Bristol-Myers Squibb Shareholding: 15,500



Hilarv Malone

Board Member (2021) COO at Valo Health (US).

Former Chief Regulatory Officer & Head of Global Regulator v Affairs at Sanofi (2013-2019)

Former SVP & Head of Worldwide Regulatory Strategy at Pfizer (2009-2011)

Chair of US Committee

Shareholding: 0

Eva Nilsagård

Board Member (2019)

Board member of several companies, e.g. Addlife, Bufab, Irras, Xbrane

Former CFO of Vitrol ife and Plasta Chair of Audit Committee

Jonas Wikström

Shareholding: 3,000



Board Member (2024) Founder/CEO of WR Capital Former fund manager at Catella Fondförvaltning, Member of Renumeration Committee Shareholding: 361,301



Board Member (2024) Managing Director, Redmile Member of Renumeration Committee Shareholding: 0















Board Member (2019) CFO of NorthSea Therapeutics Former CFOZealand Pharma Memberof Audit Committee

Shareholding: 1,000







Mats Blom









References

- 1. European Medicines Agency. Idefirix® summary of product characteristics. Available at: https://www.ema.europa.eu/en/documents/product-information/idefirix-epar-product-information_en.pdf
- Kjellman C et al. Am J Transplantation 2021 Dec;21(12):3907-3918.
- 3. Trial NCT03611621
- 4. Winstedt et al. (2015) PLOS ONE 10(7)
- 5. Lorant et al., American Journal of Transplantation and 03+04 studies (Jordan et al., New England Journal of Medicine)
- 6. Idefirix Summary of Product Characteristics. https://www.ema.europa.eu/en/documents/product-information/idefirix-epar-product-information_en.pdf. Accessed June 2024.
- 7. Reverberi R, Reverberi L. Removal kinetics of therapeutic apheresis. Blood Transfus. 2007 Jul;5(3):164-74. doi: 10.2450/2007.0032-07. PMID: 19204770; PMCID: PMC2535895.
- Hans-Hartmut Peter, MD,a Hans D. Ochs, MD,b,c Charbette Cunningham-Rundles, MD, PhD,d Donald C. Vnh, MD,e,f Peter Kiessling, PhD,g Bernhard Greve, MD,g and Stephen Jolles, MD, PhDh Freiburg and Monheim-am-Rhein, Germany, Seattle, Wash; New York, NY, Montreal, Quebec, Canada; and Cardiff, United Kingdom. Targetting FcRn for immunomodulation: Benefit, risks and practical considerations. Journal of Allergy and Clinical Immunology. 2020 September;479-9. https://www.jacionline.org/article/S0091-6749%2820%2931037-X/pdf. Accessed June 2024.
- 9. "List of Autoimmune Diseases". Autoimmune Registry Inc. https://www.autoimmuneregistry.org/autoimmune-diseases. Accessed June 4, 2024
- 10. Hellmark et al. J Autoimmun. 2014 Feb-Mar;48-49:108-1
- 11. Anti-GBM Disease. What is Anti-Glomerular Basement Membrane Disease (also known as Goodpasture's Syndrome)? https://unckidneycenter.org/kidneyhealthlibrary/glomerular-disease/anti-gbm-disease/. Accessed June 4, 2024
- 12. Hellmark et al. J Autoimmun. 2014 Feb-Mar;48-49:108-12
- 13. Sánc hez-Agesta M, et al. Front. Med (2022). 9:889185. doi: 10.3389/fmed.2022.889185
- 14. McGrogan A, et al. Neuroepidemiology. 2009; 32(2):150-63
- 15. Guillian Barre Syndrom. https://www.who.int/news-room/fact-sheets/detail/guillain-barr%C3%A9-syndrome#:~:text=Guillain%2DBarr%C3%A9%20syndrome%20(GBS),cases%20of%20Guillain%2DBarr%C3%A9%20syndrome. Accessed June 4, 2024
- 16. Fletcher D, et al. Neurology. 2000 27;54(12):2311-5
- 17. Van Doorn P. Presse Med. 2013;42(6 Pt 2):e193-201
- 18. A study of imlifidase in patients with Guillian Barre Syndrome. https://www.clinicaltrials.gov/study/NCT03943589. Accessed June 4, 2024.
- 19. Orphan Drug Designations. https://www.accessdata.fda.gov/scripts/opdlisting/oopd/detailedIndex.cfm?cfgridkey=6238. Accessed June 4 2024
- 20. The progress of the disease results in end stage kidney disease in 25 percent of patients
- 21. Falk RJ, Jennette JC. The New England journal of medicine. 1988;318(25):1651-7
- 22. Berti A, et al. Arthritis Rheum atol. 2017;69.
- 23. Rathmann J, et al. RMD Open. 2023;9:e002949.
- 24. Booth AD, et al. American journal of kidney diseases. 2003;41(4):776-84.
- 25. Samman KN, Ross C, Pagnoux C, Makhzoum JP. Update in the Management of ANCA-Associated Vasculitis: Recent Developments and Future Perspectives. Int J Rheumatol. 2021 Apr 8;2021:5534851. doi: 10.1155/2021/5534851. PMID: 33927768; PMCID: PMC8049818.
- 26. Boutin S, et al. Prevalence of serum IgG and neutralizing factors against adeno-associated virus (AAV) types 1, 2, 5, 6, 8, and 9 in the healthy population: implications for gene therapy using AAV vectors. Hum GeneTher. 2010 Jun;21(6):704-12. doi: 10.1089/hum.2009.182. PMID: 20095819.
- 27. Griffin JM, et al. Astrocyte-selective AAV gene therapy through the endogenous GFAP promoter results in robust transduction in the rat spinal cord following injury. Gene Ther. 2019 May;26(5):198-210. doi: 10.1038/s41434-019-0075-6. Epub 2019 Apr 8. PMID: 30962538; PMCID: PMC6760677.
- 28. Calcedo R, Wilson JM. Humoral Immune Response to AAV. Front Immunol. 2013 Oct 18;4:341. doi: 10.3389/fimmu.2013.00341. PMID: 24151496; PMCID: PMC3799231.
- 29. Lundstrom K. Viral Vectors in Gene Therapy. Where Do We Stand in 2023? Viruses. 2023 Mar 7;15(3):698. doi: 10.3390/v15030698. PMID: 36992407; PMCID: PMC10059137.
- 30. Mendell JR, Connolly AM, Lehman KJ, Griffin DA, Khan SZ, Dharia SD, Quintana-Gallardo L, Rodino-Klapac LR. Testing preexisting antibodies prior to AAV gene transfer therapy: rationale, lessons and future considerations. Mol Ther Methods Clin Dev. 2022 Feb 26;25:74-83. doi: 10.1016/j.omtm.2022.02.011. PMID: 35356756; PMCID: PMC8933338.
- 31. Boycott K.M, et al. Nature Reviews Genetics. 2013;14(10):681-691



References

32. Biospace https://www.biospace.com/article/releases/gene-therapy-market-size-poised-to-surge-usd-52-40-billion-by-2033/#:~: text=The%20global%20gene%20therapy%20market, period%20(2024%2D2033). Accessed June 4, 2024.

33. Pharma projects| Informa, April 2022. https://www.asgct.org/global/documents/asgct-pharma-intelligence-q1-2022-report.aspx. Accessed June 4, 2024.

34. Grand View Research, Gene Therapy Market Size, Share & Trend Analysis Report By Indication (Acute Lymphoblastic Leukemia, Large B -cell Lymphoma), By Vector Type (Lentivirus), By Route of Administration, By Region, And Segment Forecasts, 2024 – 2030. https://www.grandviewresearch.com/industry-analysis/gene-therapy-market

35. Statement from FDA Commissioner Scott Gottlieb, M.D. and Péter Marks, M.D., Ph.D., Director of the Center for Biologics E valuation and Research on new policies to advance development of safe and effective cell and gene therapies. https://www.fda.gov/news-events/press-announcements/statement-fda-commissioner-scott-gottlieb-md-and-peter-marks-md-phd-director-center-biologics. Accessed June 4, 2024

36. Rarediseases.org, https://rarediseases.org/rare-diseases/dystrophy-myotonic/ [Accessed 2023-06-28]

37. Medlineplus.gov, https://medlineplus.gov/genetics/condition/gaucher-disease/#frequency Accessed 2023-06-20

38. Sandahi TD, Laursen TL, Munk DE, Vilstrup H, Weiss KH, Ott P. The Prevalence of Wilson's Disease: An Update. Hepatology. 2020 Feb; 71(2): 722-732. doi: 10.1002/hep.30911. Epub 2020 Jan 31. PMID: 31449670.

39. Ghazi A, Grant JA. Hereditary angioe dema: epidemiology, management, and role of icatibant. Biologics. 2013;7:103-13. doi:2147/BTT.S27566. Epub 2013 May 3. PMID: 23662043; PMCID: PMC3647445

40. Hillert A, et. al The Genetic Landscape and Epidemiology of Phenylketonuria. Am J Hum Genet. 2020 Aug 6;107(2):234-250. doi: 10.1016/j.ajhg.2020.06.006. Epub 2020 Jul 14. PMID: 32668217; PMCID: PMC7413859

41. Med line plus.gov, https://medline.plus.gov/genetics/condition/fabry-disease/#frequency_Accessed: 2023-07-12.

42. Liang, WC., Jong, YJ., Wang, CH. et al. Clinical, pathological, imaging, and genetic characterization in a Taiwanese cohort with limb-girdle muscular dystrophy. Orphanet J Rare Dis 15, 160 (2020). https://doi.org/10.1186/s13023-020-01445-1

43. Rarediseases.org, https://rarediseases.org/rare-diseases/pompe-disease/ [Accessed 2023-07-12]

44. Genethon.com, https://www.genethon.com/our-pipeline/crigler-najjar-syndrome/ [Accessed 2023-06-15]

45. Gajula P, Ramalingam K, Bhadrashetty D. A rare case of mucopolysaccharidosis: Hunter syndrome. J Nat Sci Biol Med. 2012 Jan;3(1):97-100. doi: 10.4103/0976-9668.95984

46. Rarediseases.org, https://rarediseases.org/rare-diseases/omithine-transcarbamylase-deficiency/ [Accessed 2023-07-12]

47. Crisafulli S. et. Al, Global epidemiology of Duchenne muscular dystrophy: an updated systematic review and meta-analysis. Orphanet J Rare Dis. 2020 Jun 5;15(1):141. doi: 10.1186/s13023-020-01430-8. PMID: 32503598; PMCID: PMC7275323. 48. GlobalData. Accessed 2023-12-15.

49. Verhaart, I.E.C., Robertson, A., Wilson, I.J. et al. Prevalence, incidence and carrier frequency of 5q-linked spinal muscular a trophy – a literature review. Orphanet J Rare Dis 12, 124 (2017). https://doi.org/10.1186/s13023-017-0671-8

50. Burns T, Fernandez R, Stephens M. The experiences of adults who are on dialysis and waiting for a renal transplant from a deceased donor: a systematic review. JBI Database System Rev Implement Rep. 2015 Mar 12;13(2):169-211. doi: 10.11124/jbisrir-2015-1973. PMID: 26447040.

51. Organ Procurement and Transplantation Network (OPTN) and Scientific Registry of Transplant Recipients (SRTR). OPTN/SRTR 2022 Annual Data Report. U.S. Department of Health and Human Services, Health Resources and Services Administration; 2024. Accessed June 4, 2024.

52. Kjellman C et al. Am J Transplantation 2021 Dec;21(12):3907-3918.

53. Trial NCT 0361 1621

54. European Medicines Agency. Idefinix® summary of product characteristics. 55. Available at: https://www.ema.europa.eu/en/documents/product-information/idefinix-epar-product-information_en.pdf

55. OPTN National Data, https://optn.transplant.hrsa.gov/data/view-data-reports/national-data/

56. Global Observatory on Donation and Transplantation, https://www.transplant-observatory.org/ [Accessed 2024-01-07]

57. Appel JZ 3rd, Hartwig MG, Davis RD, Reinsmoen NL. Utility of peritransplant and rescue intravenous immunoglobulin and extracorporeal immunoadsorption in lung transplant recipients sensitized to HLA antigens. Hum Immunol. 2005;66:378–86.

58. Levine DJ, Glanville ÅR, Aboyoun C, et al. Antibody-mediated rejection of the lung: a consensus report of the International Society for Heart and Lung Transplantation. J Heart Lung Transplant. 2016;35:397–406.

59. Roux A, Bendib Le Lan I, Holifanjaniaina S, et al. Antibody-mediated rejection in lung transplantation: clinical outcomes and donor-specific antibody characteristics. Am J Transplant. 2016; 16:1216–28.

60. Post-Transplant Outcome of the Highly Sensitized Patient Awaiting Heart Transplant Treated with Desensitization. Kobashigawa, J.A. et al. The Journal of Heart and Lung Transplantation, Volume 40, Issue 4, S44.

61. Chih S, Chruscinski A, Ross HJ, Tinckam K, Butany J, Rao V. Antibody-mediated rejection: an evolving entity in heart transplantation. J Transplant. 2012;2012:210210. doi: 10.1155/2012/210210. Epub 2012 Mar 26. PMID: 22545200; PMID: PMC3321610.

