## Rare Diseases Pipeline

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The agents mentioned here are investigational and have not been approved by any regulatory agency worldwide for the uses under investigation.

ASMD, acid sphingomyelinase deficiency; FGFR3, fibroblast growth factor receptor 3; GD1, Gaucher disease type 1; GD3, Gaucher disease type 3; IOPD, Infantile-onset Pompe disease; miRNA, microRNA.
Avalglucosidase alfa is an investigational recombinant acid alpha-glucosidase (GAA) for IOPD.

Carbohydrate groups on the avalglucosidase alfa molecule bind to mannose-6-phosphate receptors (M6P).

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

GAA, acid alpha-glucosidase; IOPD, Infantile-onset Pompe disease; M6P, mannose-6-phosphate

The agents mentioned are investigational for the indications stated and have not been approved by any regulatory agency worldwide for the uses under investigation.
Avalglucosidase alfa is an investigational recombinant acid alpha-glucosidase (GAA) for IOPD.

Increased M6P moieties on the avalglucosidase alfa molecule bind to M6P receptors.

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

GAA, acid alpha-glucosidase; IOPD, Infantile-onset Pompe disease; M6P, mannose-6-phosphate

The agents mentioned are investigational for the indications stated and have not been approved by any regulatory agency worldwide for the uses under investigation.
Proposed mechanism of action

Avalglucosidase alfa is transported into the cell and delivered to the lysosome.

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

IOPD, Infantile-onset Pompe disease

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**Proposed mechanism of action**

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IOPD, Infantile-onset Pompe disease

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Proposed mechanism of action

Avalglucosidase alfa helps cleave glycogen to glucose.

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IOPD, Infantile-onset Pompe disease

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Proposed mechanism of action

Avalglucosidase alfa helps cleave glycogen to glucose.

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IOPD, Infantile-onset Pompe disease

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Proposed mechanism of action

Avalglucosidase alfa helps cleave glycogen to glucose, decreasing the glycogen storage.

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IOPD, Infantile-onset Pompe disease

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Proposed mechanism of action

Acid sphingomyelinase breaks down sphingomyelin to ceramide and phosphocholine.

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

ASMD, acid sphingomyelinase deficiency.

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Proposed mechanism of action

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ASMD, acid sphingomyelinase deficiency.

The agents mentioned are investigational for the indications stated and have not been approved by any regulatory agency worldwide for the uses under investigation.
Olipudase alfa is designed to enter the lysosome.

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**Proposed mechanism of action**

Olipudase alfa is designed to enter the lysosome.

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

ASMD, acid sphingomyelinase deficiency.

The agents mentioned are investigational for the indications stated and have not been approved by any regulatory agency worldwide for the uses under investigation.
Proposed mechanism of action

Olipudase alfa is designed to act like endogenous acid sphingomyelinase to supplement the breakdown of sphingomyelin to ceramide and phosphocholine.

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

ASMD, acid sphingomyelinase deficiency.

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For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

ASMD, acid sphingomyelinase deficiency.

The agents mentioned are investigational for the indications stated and have not been approved by any regulatory agency worldwide for the uses under investigation.
An open label, two-cohort (with and without imiglucerase), multicenter study to evaluate pharmacokinetics, safety, and efficacy of eliglustat in pediatric patients with Gaucher disease type 1 and type 3

**Primary outcome**
- PK assessment: $C_{\text{max}}$ and AUC
- Adverse events

**Secondary outcomes**
- QoL measured by PedsQL
  - Cohort 1:
    - Change in hemoglobin level
    - Change in platelet count
    - Change in liver and spleen volume
  - Cohort 2:
    - Pulmonary disease improvement
    - Bone disease improvement
    - Thrombocytopenia improvement

**Study design**

**Cohort 1**: Eliglustat monotherapy, oral
- Rescue treatment (if significant clinical decline):
  - Step 1: Switch from eliglustat to imiglucerase monotherapy
  - Step 2: Combination therapy with eliglustat + imiglucerase if 6 months without improvement using imiglucerase monotherapy

**Cohort 2**: Eliglustat, oral + imiglucerase, infusion at dose prior to enrollment
- After 52 weeks:
  - Switch to eliglustat monotherapy if desired clinical response achieved

**Patient population**
- N=60
- Pediatric patients diagnosed with Gaucher disease type 1 or 3

**Enrolling countries**
- ELIKIDS (Phase 3 study)

**Proposed mechanism of action**

**ELIKIDS (Phase 3 study)**

**Study design**

**Proposed mechanism of action**

**Patient population**

**Enrolling countries**

**References**

The agents mentioned are investigational for the indications stated and have not been approved by any regulatory agency worldwide for the uses under investigation.

AUC, area under the curve; $C_{\text{max}}$, maximum concentration; PedsQL, Pediatric Quality of Life Inventory; PK, pharmacokinetic; QoL, quality of life
Proposed mechanism of action

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

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Eliglustat
Gaucher disease type 1 & 3

ELIKIDS (Phase 3 study)

Study design

Patient population

Enrolling countries
Patient population

All patients:
• Aged 2 to <18 years at time of informed consent, with clinical diagnosis of Gaucher disease type 1 or 3
• Negative pregnancy test at screening and baseline
• No substrate reduction therapy for GD within 6 months prior to enrollment
• No partial or total splenectomy if performed within 2 years prior to enrollment
• No neurological symptoms other than oculomotor apraxia at study entry
• No history of esophageal varices, liver infarction, or elevated liver enzymes
• No significant cardiovascular conditions
• Not a CYP2D6 ultra-rapid metabolizer or indeterminate metabolizer

Cohort 1 (eliglustat monotherapy):
• Receiving ERT for ≥ 24 months at a monthly dose of 30 U/kg to 130 U/kg of imiglucerase
• At pre-specified treatment goals

Cohort 2 (eliglustat + imiglucerase):
• Receiving ERT for ≥ 36 months at a dose equivalent to at least 60 U/kg of imiglucerase every 2 weeks
• A dose stable for at least the 6 months preceding enrollment.
• Severe clinical manifestations of GD (pulmonary disease, symptomatic bone disease, or persistent thrombocytopenia)

Only key inclusion/exclusion criteria are listed; please visit ClinicalTrials.gov (NCT03485677) for further details.

ERT, enzyme replacement therapy; GD, Gaucher disease

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Eliglustat
Gaucher disease type 1 & 3

ELIKIDS (Phase 3 study)

Study design
Proposed mechanism of action
Patient population

Enrolling countries

The agents mentioned are investigational for the indications stated and have not been approved by any regulatory agency worldwide for the uses under investigation.
Randomized, double-blind, placebo-controlled study to assess the efficacy, pharmacodynamics, pharmacokinetics, safety and tolerability of venglustat in patients with late-onset GM2 gangliosidosis together with an open-label study in other similar ultrarare diseases.

**Study design**

**Primary population**

- N=57
- Adults with late-onset GM2 gangliosidosis (Tay-Sachs and Sandhoff disease)

R: 2:1

- Venglustat QD, fixed dose
- Placebo QD

**Secondary population**

- N=20
- Patients ≥2 years of age with GM2 gangliosidosis, and other similar ultrarare diseases

- Venglustat QD at various doses

**Primary outcomes**

- **Primary population**
  - Change in CSF GM2
  - Change in 9-hole peg test

- **Secondary population**
  - Pharmacodynamic responses in plasma and CSF

**Secondary outcomes**

- Adverse events
- Pharmacokinetic parameters in plasma and CSF (C\text{max}, t\text{max}, AUC\text{0–24h})
- Change in 25-foot walk test
- Change in Friedrich’s Ataxia Rating Scale (FARS)

**Secondary population**

- Change in 9-hole peg test

**Study design**

- *Planned enrollment
- †Juvenile/adolescent GM2 gangliosidosis, GM1 gangliosidosis, saposin C deficiency, sialidosis type 1 or juvenile/adult galactosidosis.

- AUC\text{0–24h}, area under curve from 0 to 24 hours; C\text{max}, maximum concentration in plasma; CSF, cerebrospinal fluid; FARS, Friedrich’s Ataxia Rating Scale; QD, once daily; R, randomization; t\text{max}, time to maximum concentration.

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**References**

N=20 MAT-US-2100084, v4.0 Exp date: 10/5/2023
Proposed mechanism of action

GCS converts ceramide into GL-1.

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

GCS, glucosylceramide synthase; GL-1, glucosylceramide.

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Venglustat
GM2 gangliosidosis

AMETHIST (Phase 3 study)
Study design
Proposed mechanism of action
Patient population
Enrolling countries

**Proposed mechanism of action**

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References
Proposed mechanism of action

Venglustat
GM2 gangliosidosis

AMETHIST (Phase 3 study)

Study design

Patient population

Enrolling countries

GCS converts ceramide into GL-1.

GL-1 is a key substrate for biosynthesis of glycosphingolipids, accumulation of which is implicated in disease pathophysiology.

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Proposed mechanism of action

Venglustat is designed to block the active site of GCS and to decrease synthesis of GL-1.

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

GCS, glucosylceramide synthase; GL-1, glucosylceramide.

The agents mentioned are investigational for the indications stated and have not been approved by any regulatory agency worldwide for the uses under investigation.
Patient population

Primary population

- Aged ≥18 years
- Late-onset GM2 gangliosidosis (Tay-Sachs disease or Sandhoff disease) caused by genetic β-hexosaminidase deficiency resulting from mutations in HEXA or HEXB
- Ability to perform the 9-HPT at screening visit in ≤ 240 seconds for the 2 consecutive trials of both hands
- Additional criteria required for all patients, see below

Secondary population

- Adult population: Aged ≥18 years
- Juvenile population: Aged ≥2 years and < 18 years with weight ≥ 10 kg
- Diagnosis of juvenile/adolescent GM2 gangliosidosis, GM1 gangliosidosis, saposin C deficiency, sialidosis type 1 or juvenile/adult galactosialidosis
- Additional criteria required for all patients, see below

All patients

- If there is a history of seizures, they must be well controlled by medication (not strong/moderate inducer or inhibitors of CYP3A4)
- No use of invasive ventilatory support
- No cortical or posterior subcapsular cataracts grade ≥ 2 (nuclear cataracts are allowed)
- ALT/AST or total bilirubin ≤ 2x ULN unless diagnosed with Gilbert syndrome
- eGFR ≥ 30 mL/min/1.73m²

Only key inclusion/exclusion criteria are listed; please visit ClinicalTrials.gov (NCT04221451) for further details.

9-HPT, 9-Hole Peg Test; ALT, alanine aminotransferase; AST, aspartate aminotransferase; eGFR, estimated glomerular filtration rate; ULN, upper limit of normal

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Study design

Proposed mechanism of action

Venglustat
GM2 gangliosidosis

AMETHIST (Phase 3 study)

Patient population

Enrolling countries

USA, United States of America.

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Proposed mechanism of action

Venglustat is designed to block the active site of GCS and to decrease synthesis of GL-1.

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

GCs, glucosylceramide synthase; GL-1, glucosylceramide.

The agents mentioned are investigational for the indications stated and have not been approved by any regulatory agency worldwide for the uses under investigation.
Randomized, double-blind, placebo-controlled study to evaluate the safety, efficacy, pharmacodynamics, and pharmacokinetics of Lademirsen for weekly subcutaneous injection in patients with Alport syndrome.

**Study design**

**Primary outcome**
- Number of participants with adverse events (up to 106 weeks)
- Annualized change in eGFR from baseline to Week 48

**Secondary outcomes**
- Pharmacokinetics: $C_{\text{max}}$ and $C_{\text{trough}}$
- Number of participants with anti-drug antibodies (ADAs)
- Number of participants with adverse events related to ADAs
- Percent change in eGFR values
- Proportion of participants reaching end-stage renal disease at 48 weeks
- Pharmacodynamic effects on miR-21 and on changes in renal injury and function biomarkers

*Estimated enrollment.

$C_{\text{max}}$, maximum concentration in plasma; $C_{\text{trough}}$, trough plasma concentration; eGFR, estimated glomerular filtration rate; R, randomization.

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Proposed mechanism of action

miR-21 is a type of microRNA, a short, non-coding RNA.

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

RNA, ribonucleic acid

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References
Proposed mechanism of action

It binds to messenger RNA...

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

RNA, ribonucleic acid

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Proposed mechanism of action

...blocking the ribosome and preventing protein expression.

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

RNA, ribonucleic acid

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Proposed mechanism of action

Lademirsen is designed to bind to miR-21 ...

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

RNA, ribonucleic acid

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Lademirsen
Alport syndrome

HERA (Phase 2 study)

Study design

Proposed mechanism of action

...preventing it from acting on the messenger RNA.

For illustrative purposes only. The clinical significance of this mechanism of action is under investigation.

RNA, ribonucleic acid

The agents mentioned are investigational for the indications stated and have not been approved by any regulatory agency worldwide for the uses under investigation.
Patient population

- Aged 18 to 55 years
- Alport syndrome confirmed by clinical diagnosis (hematuria, family history, hearing loss, ocular changes) AND genetic confirmation in the subject or family member OR kidney biopsy showing glomerular basement membrane abnormalities consistent with Alport syndrome*
- eGFR >35 and <90 mL/min/1.73m²/year at screening
- At least one of the following clinical markers of disease progression:
  - Decline in eGFR ≥4 mL/min/1.73m²/year (minimum of 2-year time span)
  - Proteinuria defined as urine protein:creatinine ratio >2,000 mg/g
  - Age and sex-adjusted eGFR (based on CKD-Epi; male with an eGFR <90 mL/min/1.73m² at 18-23 years of age)
- ACEi and/or ARB dose stable for at least 30 days prior to screening
- No treatment with bardoxolone within 90 days prior to screening
- No history of kidney disease aside from Alport syndrome, kidney transplantation or dialysis

*E.g. significant thinning, thickening, irregularity, or lucencies.

Only key inclusion/exclusion criteria are listed; please visit ClinicalTrials.gov (NCT02855268) for further details.

ACEi, angiotensin-converting enzyme inhibitor; ARB, angiotensin receptor blocker; eGFR, estimated glomerular filtration rate.

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Lademirsen
Alport syndrome

HERA (Phase 2 study)
Study design
Proposed mechanism of action
Patient population

Enrolling countries

USA, United States of America

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References