

META+ Research Areas of Interest

Neurofibromatosis type 1 plexiform neurofibromas (NF1-PN)

- Early use of selumetinib in pediatric NF1-PN patients prior to emergence of severe symptoms and need for surgery
- Studies exploring re-treatment with selumetinib in pediatric NF1-PN patients who have discontinued therapy (for reasons other than toxicity or progression) for >3 months
- Pre- or post-use of selumetinib in combination with debulking surgery in pediatric NF1-PN patients
- Real world evidence in adult NF1-PN population

Out of Scope

- Selumetinib treatment in non-rare tumors.
- Selumetinib treatment for hematological malignancies

Lysosomal acid lipase deficiency (LAL-D)

- Diagnosis and differential diagnosis of LAL-D, including familial hypercholesterolemia, fatty liver disease, secondary HLH and failure to thrive
- Treatment options, including specific dietary care, employed for the treatment of signs and symptoms of LAL-D
- Burden of disease of LAL-D on patients, care givers, families and society
- Fatty liver disease in conjunction with low LAL activity

Out of Scope

- Interventional studies for other indications

Hypophosphatasia (HPP)

- Diagnostic pathways for HPP in children and adults
- Burden of disease and disease course in HPP patients across all age groups, with both skeletal and non-skeletal manifestations (e.g. muscle weakness)
- Real world effectiveness and safety of asfotase alfa in HPP