Cincinnati Children's | Innovation Ventures

Genetics Immunology

Complement 5a increases inflammation in Hunter Syndrome

Brief Description of Technology

Complement 5a increases inflammation in Hunter Syndrome

TECHNOLOGY ID

2018-0105

COMPLEMENTARY TECHNOLOGY

2018-0205

BUSINESS OPPORTUNITY

Exclusive License or Sponsored Research

TECHNOLOGY TYPE

Small Molecule

PATENT INFORMATION

Provisional Filed

LEARN MORE

Innovation Ventures
partnering@cchmc.org
1.513.636.4285

innovation.cincinnatichildrens.org

Technology Overview

Iduronate-2-sulfatase defect and resultant excess tissue accumulation of glycosaminoglycans (GAGs), are the hall mark of Hunter's syndrome (HS). Affected individuals exhibit inflamed organs with marked increases of immune inflammation that lead to early death. The mechanism by which GAGs propagate in HS is lacking. We have recently identified that glucosylceramide induced excess generation of C5a sparks immune inflammation in Gaucher disease (Nature 2017). To determine whether this axis is also involved in immune inflammation in other lysosomal storage diseases, we have used HS patients and control samples. Results showed HS-mice and HS patients showed elevated level of C5a and C5aR1.

Applications

Targeting C5a-C5aR1 axis will reduce clinical symptoms of Hunter Syndrome

Advantages

- Potentially provide treatment options for diseases with “sequestered or untreated” aspects of lung and brain defects.

Market Overview

The cost to treat an individual with enzyme replacement (e.g., elaprase) is significant, about $300,000- 500,000 / per year. Development of alternative effective therapies have been hampered by limitations in understanding of disease pathogenesis and toxicity concerns due to the blood brain barrier and procedural risks. Thus, there is an need for better therapeutic approaches for Hunter Syndrome.

Investigator Overview

Manoj Pandey, PhD, Division of Human Genetics