**Genetics Immunology**

**Complement 5a increases inflammation in Hunter Syndrome**

**Brief Description of Technology**

Complement 5a increases inflammation in Hunter Syndrome

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**Technology Overview**

Iduronate-2-sulfatase defect and resultant excess tissue accumulation of glycosaminoglycans (GAGs), are the hallmark 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**

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