Engineering B cells to secrete select antibodies and gene products for the treatment of diseases

**Business Opportunity**
- Exclusive license
- Non-exclusive license
- Sponsored Research

**Technology Type**
- Gene Therapy
- Therapeutic

**State of Development**
- Preclinical in vitro
- Preclinical in vivo

**Patent Information**
- Patent pending

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**Brief Description**
Method to produce B cells that can provide prolonged and tunable expression of select antibodies and gene products for the treatment of diseases (for e.g., lysosomal storage diseases)

**Technology Overview**
Several medical disorders are caused by an insufficiency of a gene product or a defective gene product. For e.g., lysosomal storage diseases are inherited metabolic disorders characterized by lack of sufficient lysosomal enzymatic activity, leading to abnormal accumulation of specific macromolecules that can lead to organ dysfunction or even organ failure. Enzyme replacement therapy (ERT) can be used as a symptomatic treatment. While beneficial, ERT is cumbersome as it requires patients to have bi-weekly IV infusion of the enzyme for the lifetime of the patient, affecting their quality of life. Thus, better treatment options are needed. To address this, Fred Hutchinson Cancer Center researchers have developed methods to engineer B cells to express a selected antibody and a gene product (secreted and non-secreted). The expression of the gene product is tunable and can be upregulated or downregulated to achieve safe, optimal and prolonged expression of the therapeutic product in-vivo.

**Applications**
- Treatment of lysosomal disorders (e.g., Gaucher disease, Fabry disease, Pompe disease etc.)
- Treatment of clotting disorders (e.g., hemophilia)
- Treatment of enzyme deficiencies (e.g., hemolytic anemia / Glucose-6-phosphate dehydrogenase or pyruvate kinase deficiency)

**Advantages**
- Obviates the need for biweekly enzyme replacement therapy infusions
- Allows tunable expression and safeguards on expression thresholds