

## HUMAN MONOCLONAL ANTIBODIES TARGETING GLYPICAN-2 IN NEUROBLASTOMA

### SUMMARY

Researchers at the National Cancer Institute's Laboratory of Molecular Biology (NCI LMB) have developed and isolated several single domain monoclonal human antibodies against GPC2. NCI seeks parties interested in licensing or co-developing GPC2 antibodies and/or conjugates.

### REFERENCE NUMBER

E-211-2016

### PRODUCT TYPE

- Therapeutics

### KEYWORDS

- Glypican-2, GPC2, Antibody, Immunotoxin, Recombinant Immunotoxin, RIT, Chimeric Antigen Receptor, CAR, Antibody-drug Conjugate, ADC, bispecific antibody, neuroblastoma

### COLLABORATION OPPORTUNITY

This invention is available for licensing and co-development.

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### DESCRIPTION OF TECHNOLOGY

Neuroblastoma is a rare pediatric cancer that affects one in every hundred thousand children under the age of fifteen in the United States. Current standards of care are chemotherapy and surgery, followed by stem-cell treatments, radiation and anti-ganglioside antibody therapy, which yield an average three-year survival rate of 10-45%. This demonstrates a need for more effective therapies.

Glypican-2 (GPC2) is a cell surface protein that has been shown to be preferentially expressed on numerous pediatric cancers, including neuroblastoma. Due to this preferential expression, GPC2 represents a potential candidate for targeted therapy.

Researchers at the National Cancer Institute's [Laboratory of Molecular Biology](#) (NCI LMB) have developed and isolated several single domain monoclonal human antibodies against GPC2. This technology covers the naked GPC2 antibodies as well as their use as targeting domains in recombinant immunotoxins (RITs) and chimeric antigen receptors (CARs). RITs (using clones LH1, LH4, or LH7) and

CARs (using LH7) have shown specific killing activity against GPC2-expressing cells, suggesting that these candidates may be further developed as therapeutics.

The technology has been validated with *in-vitro* studies (human anti-GPC2 RITs and CARs can bind to, and kill, GPC2-positive tumor cells) and the researchers are currently developing mouse models to further develop GPC2-targeted therapies.

#### Development Stage:

*In-vitro*

### POTENTIAL COMMERCIAL APPLICATIONS

- Therapeutic applications include unconjugated antibodies and use as targeting moieties for immunoconjugates such as CARs, ADCs, immunotoxins, and bispecific antibodies
- Diagnostic agent for detecting and monitoring target-expressing malignancies

### COMPETITIVE ADVANTAGES

- First to market potential – No current clinical trials with GPC2-targeted therapies
- Human antibody with high specificity and binding to targets results in less non-specific cell killing, therefore fewer potential side-effects for the patient
- Small size of single domain antibodies enhances stability, solubility, and target recognition

### INVENTOR(S)

Mitchell Ho (NCI), *et al.*

### DEVELOPMENT STAGE

- Discovery (Lead Identification)

### PATENT STATUS

- **U.S. Provisional:** US Provisional Application 62/369,861 (HHS Reference No. E-211-2016/0-US-01) filed August 2, 2016, entitled “Human Monoclonal Antibodies Targeting Glypican-2 in Neuroblastoma”

### THERAPEUTIC AREA

- Cancer/Neoplasm