

Biotechnology, Life Science, autoimmune disease,  
method,

## A method to identify modulators for regulatory T cell functions

Docket #T4518

### STATE OF DEVELOPMENT

Elucidation of mechanism of action in an in vitro model system of human cells from with XLAAD/IPEX patients.

### INTELLECTUAL PROPERTY

US Patent 8,846,308.

### REFERENCE MEDIA

Li, B et al. International Immunology 19.7 (2007): 825-35.

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### Technology Overview

Maintenance of tolerance to self-antigens is essential for the prevention of autoimmunity. This process is known to involve specific regulatory T cells (Treg) and the expression of the forkhead family transcription factor, FOXP3. FOXP3 is a “sufficient” regulator of the development and function of peripheral Treg cells, but the molecular mechanisms of FOXP3-mediated immunological regulation are still poorly understood.

Mutations in the forkhead domain of FOXP3 are found in the fatal recessive disorder, “X-linked autoimmunity and allergic dysregulation syndrome” (XLAAD) or “Immunodysregulation, polyendocrinopathy and enteropathy, X-linked syndrome” (IPEX). These individuals fail to develop CD4+CD25+ T cells and experience varied symptoms from insulin-dependent diabetes to anemia, as well as massive T cell infiltration of the skin and gastrointestinal tract.

The present invention features methods of identifying immune response modulators by measuring various aspects of FOXP3 function.

### Advantages

- An approach to identify novel inhibitors for diseases that regulatory T cells are involved.
- Specific to FOXP3 functions that are relevant to the binding to HDAC and HAT.

### Inventor

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