



Transposagen

Better Models. Better Results™

Genetic Modification for Better Disease Models™

Jack Crawford MS, VP Business Development





About Us

Transposagen

Technologies:

- *piggyBac*[™]
- XTN[™] TAL Target-Specific Nucleases
- Rodent Stem Cells

Applications:

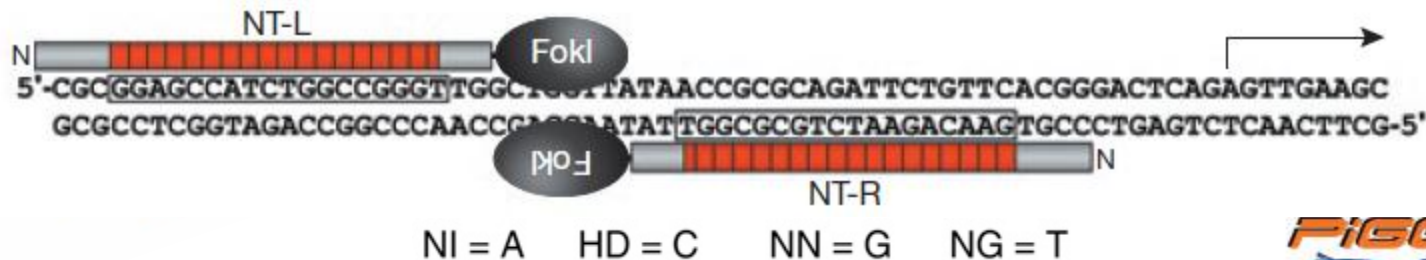
- Reagents
- Rodent Model Creation
- Cell Line Engineering & Stem Cells
- Biologics Production
- Therapeutics

Transposagen Biopharmaceuticals, Inc., a Lexington, KY-based company, is a worldwide leader in technologies and services for genetic modification. Transposagen is dedicated to providing better disease models through genetic modification. Transposagen specializes in custom and off-the-shelf XTN[™] site-specific nucleases, animal models, cell lines, stem cells and cutting-edge research tools and technologies to improve drug discovery and development research. These unique technologies and services for genetic modification give customers the ability to create nearly any genetic modification in any genome for both research and protein production purposes.



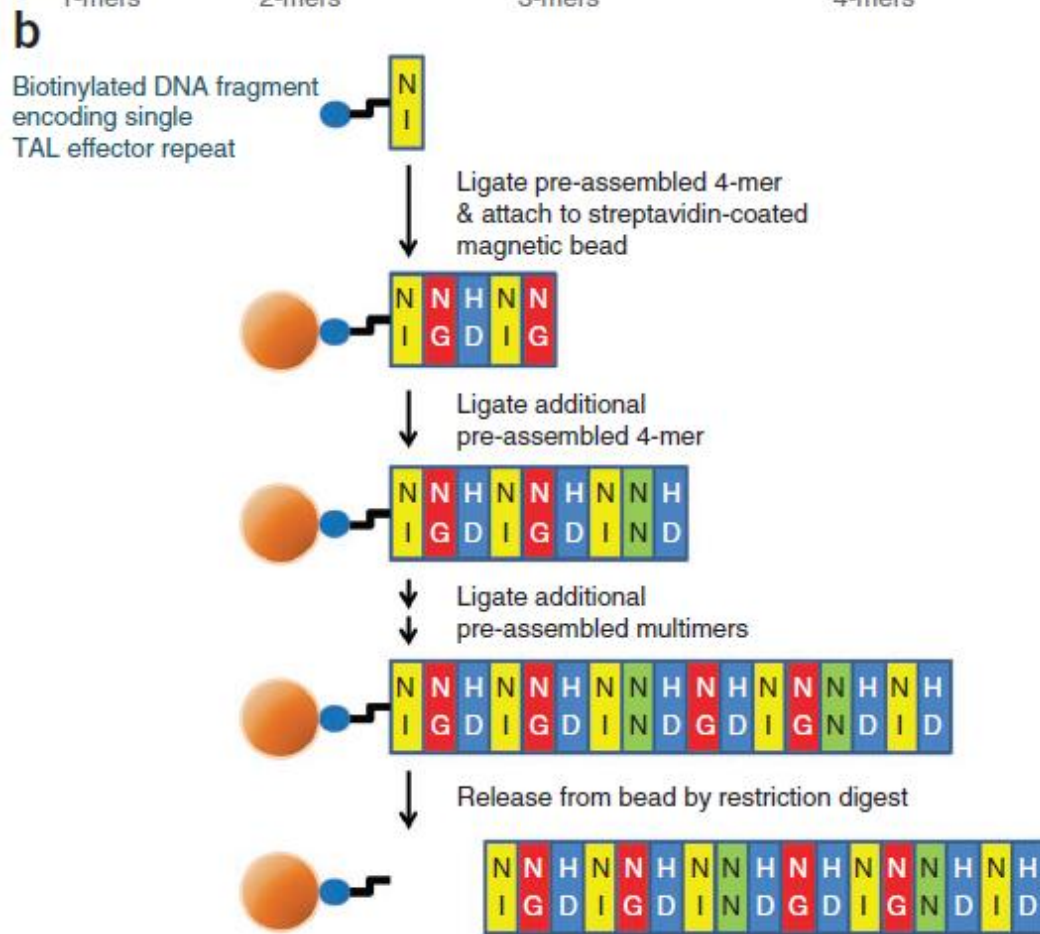
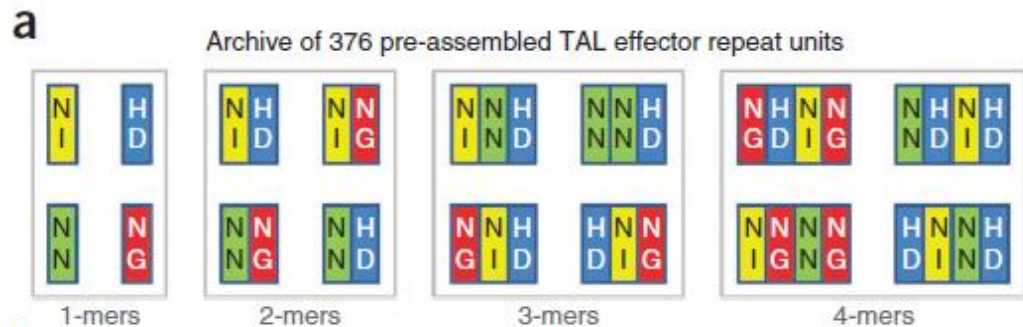
Site Specific Genetic Engineering

- Xanthomonas TAL nuclease (XTN: a.k.a TALENs)
- RVD's (Repeat Variable Disresidues)
- TAL DNA binding modules and FokI DNA-cleavage domain
- Site specific knockout, knock-in, single base-pair edits of any gene, any genome
- Automated synthesis of XTNs
- Remove transposon after gene editing

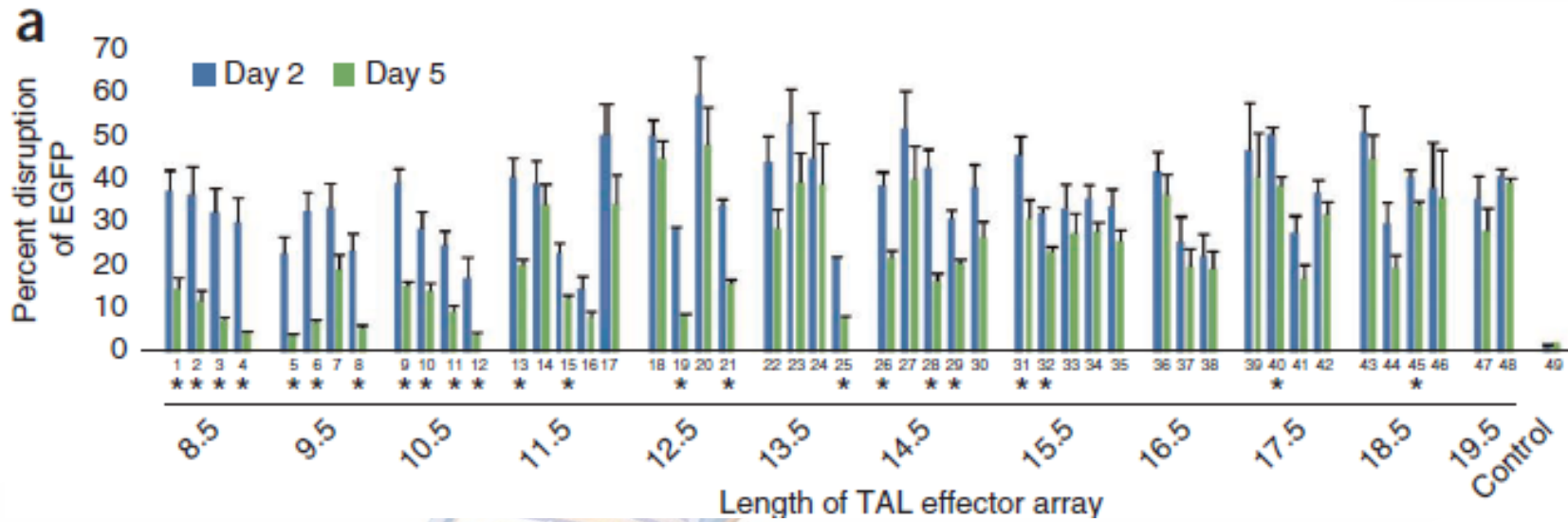


FLASH™ TAL Assembly

- Archive 376 plasmids encoding all possible combinations of the RVDs
- Automation of serial restriction digest
- No requirement for PCR, gel isolation of fragments and/or passage and characterization of intermediate constructs
- 96 well robotic format produces 96 TAL repeats in a single day



100% Knockout Efficiency

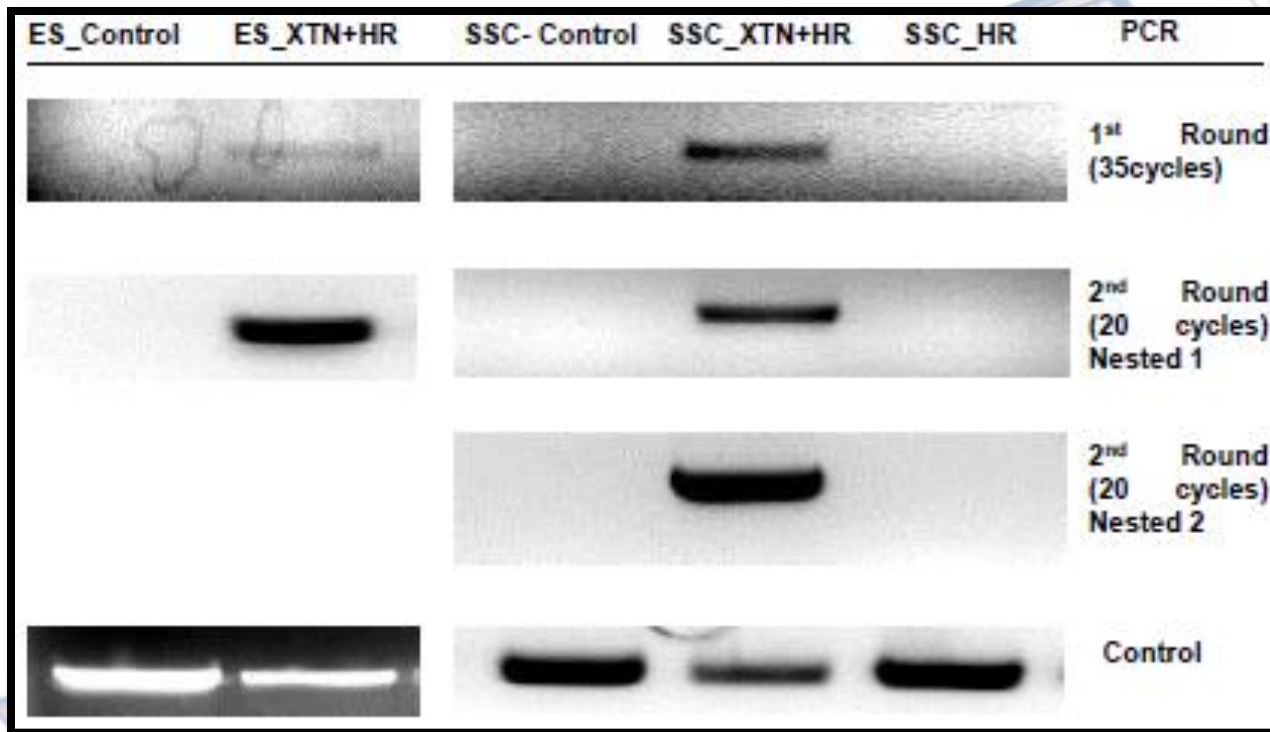


- 48 FLASH assembled TALs targeting EGFP

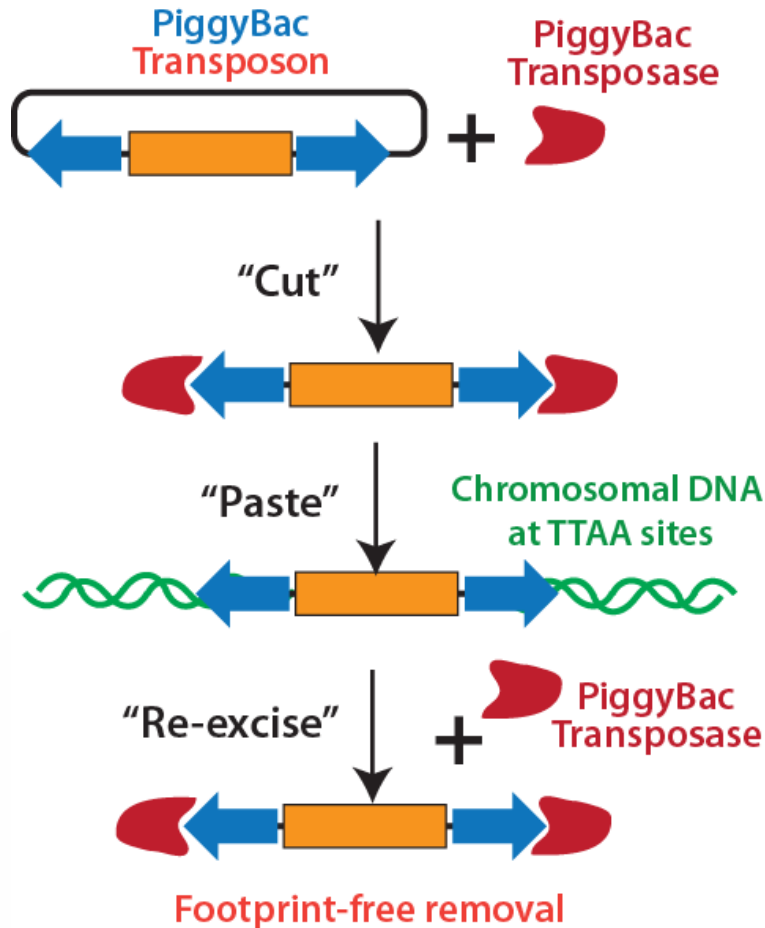


Site Specific Knock-in

- A. Site specific binding and induced dsDNA breaks
- B. Transfect SSCs
- C. Nested PCR confirmation of Homologous Recombination events



The piggyBac™ Transposon System

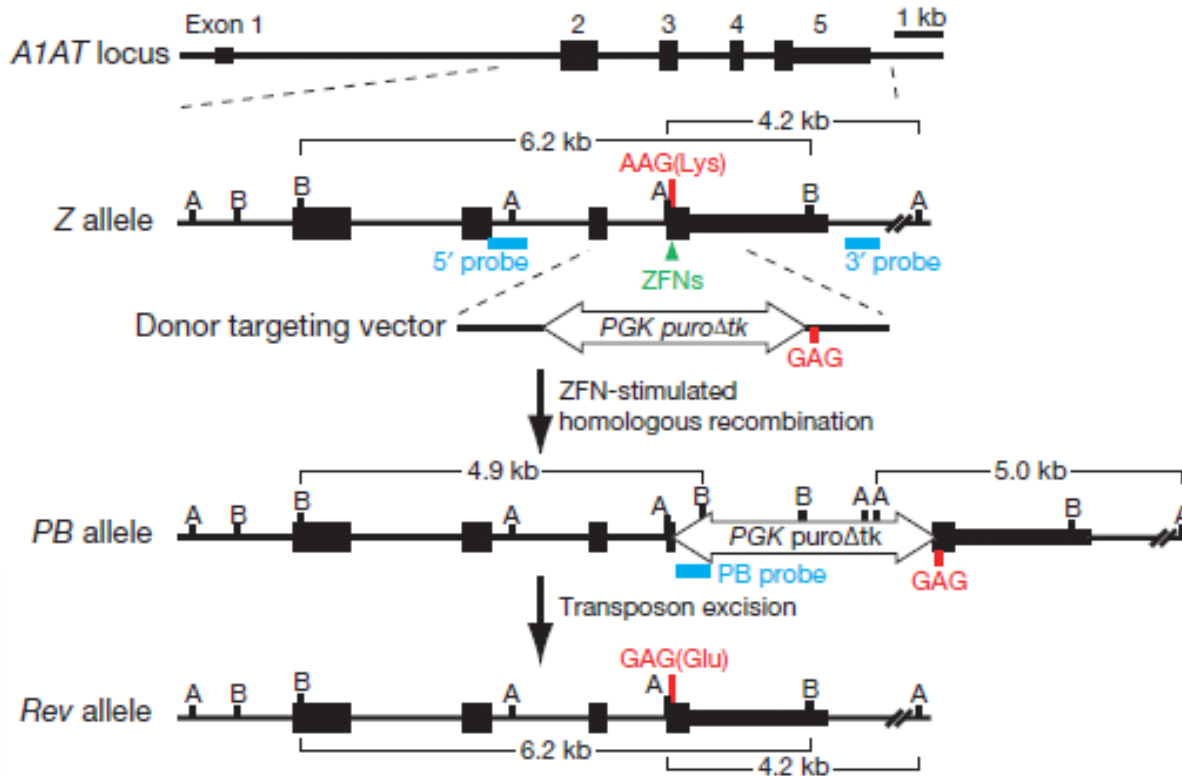


Key Features

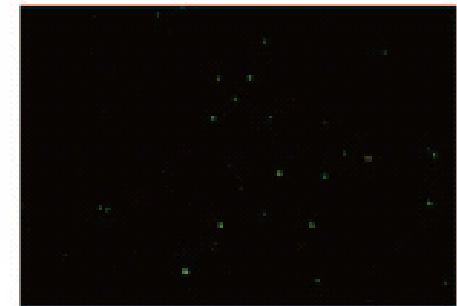
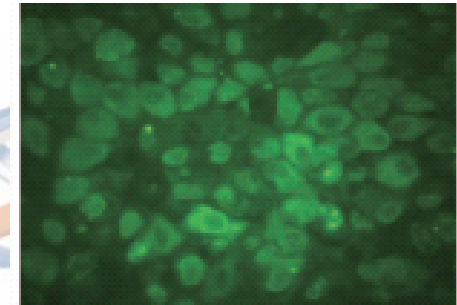
- Efficient transgenesis using transfection
- Titratable number of integrations
- Large cargo limit – 10 to 100kb
- Rapid identification of integration site
- Reversible transposition – no footprint
- All-in-one inducible vector
- Safe non-viral system



Targeted Integration and Excision



Polymeric A1AT



Animal Models
Therapeutics
Cell Lines

Bradley et al Nature (2011) In Press



Footprint-Free™ Genetic Engineering

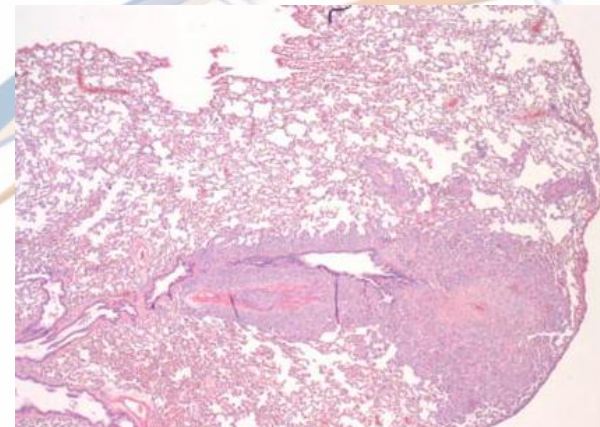
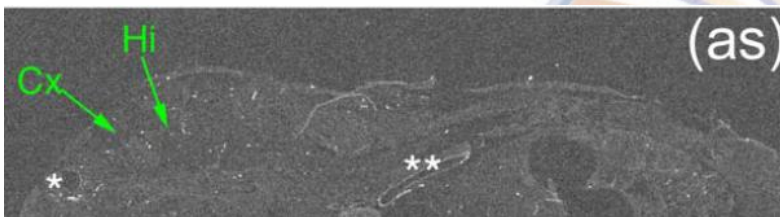
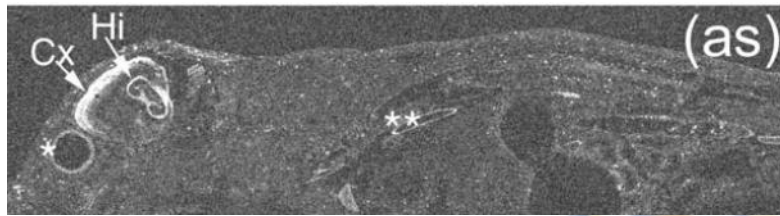


- Gene Editing Vectors
- Cell Lines & *In Vivo* Models
- Custom synthetic homology arms
- TK selection for removal of the cassette



Animal Models

- Knockout
- Transgenic Overexpression
- Conditional
- Knock-in
- Transgenic knockdown
- Humanized



Rat and mouse models available
Any species possible

Custom Services Overview

- **Vector creation: Gene editing with XTNs (talens), HR vector, piggyBac Transposon**
 - Site specific knockout, knock-in, edit and correction of any gene in any genome
- **Stable cell lines**
 - Overexpression, knockdown, reporter lines, iPS reprogramming, iPS and ES differentiation and selection, therapeutic applications
- **Animal models**
 - Rat and mouse model generation available, additional species possible

