

2017: A YEAR IN REVIEW

A Look at the Work of the
Boards in 2017

YEAR IN REVIEW: In 2017, the Boards continued to push boundaries via innovation in **quantity**, **content**, and **method** of ethical review.

HIGH NUMBER OF REVIEWS: Boards reviewed more studies than national average

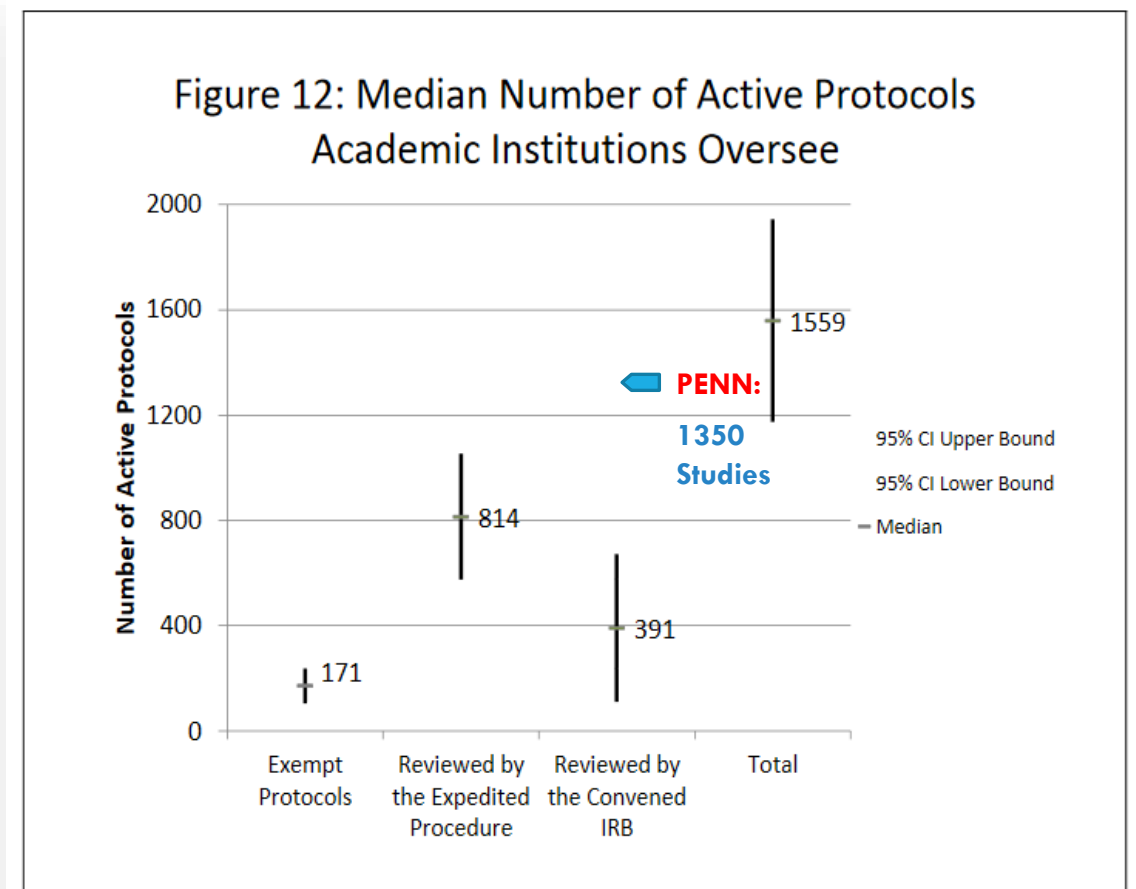
INNOVATIVE STUDIES: survey the types of studies Boards assessed this year

REVOLUTIONARY in METHODS: steps taken on

UNPARALLELED QUANTITY:

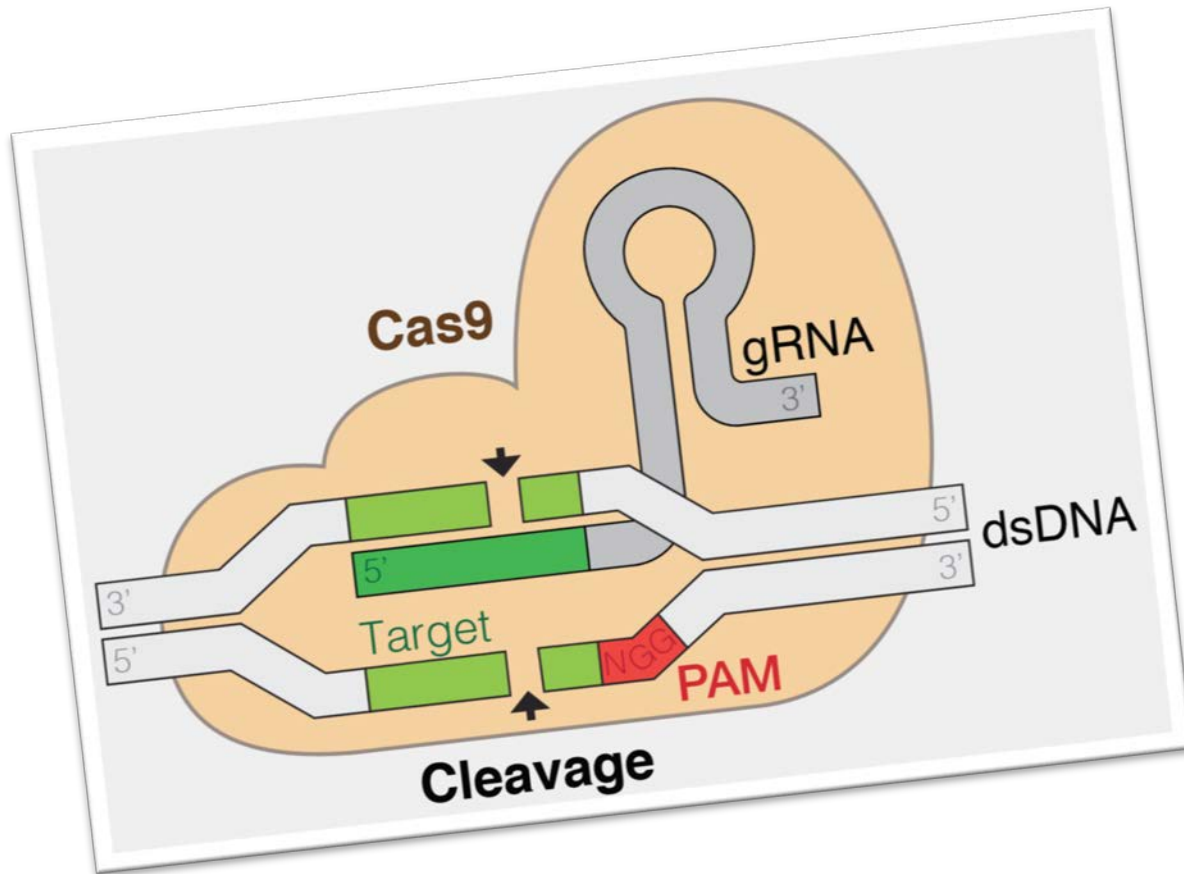
In 2017, the Penn Boards are **above the 95th percentile** of IRBs nationally.

- According to the most recent AAHRPP evaluations, the **median IRB** oversees **391** studies at the convened level
- By comparison, in the past year, the Boards have collectively overseen **1350** new & continuing studies at Penn



STUDIES REVIEWED & NOW AT PENN:

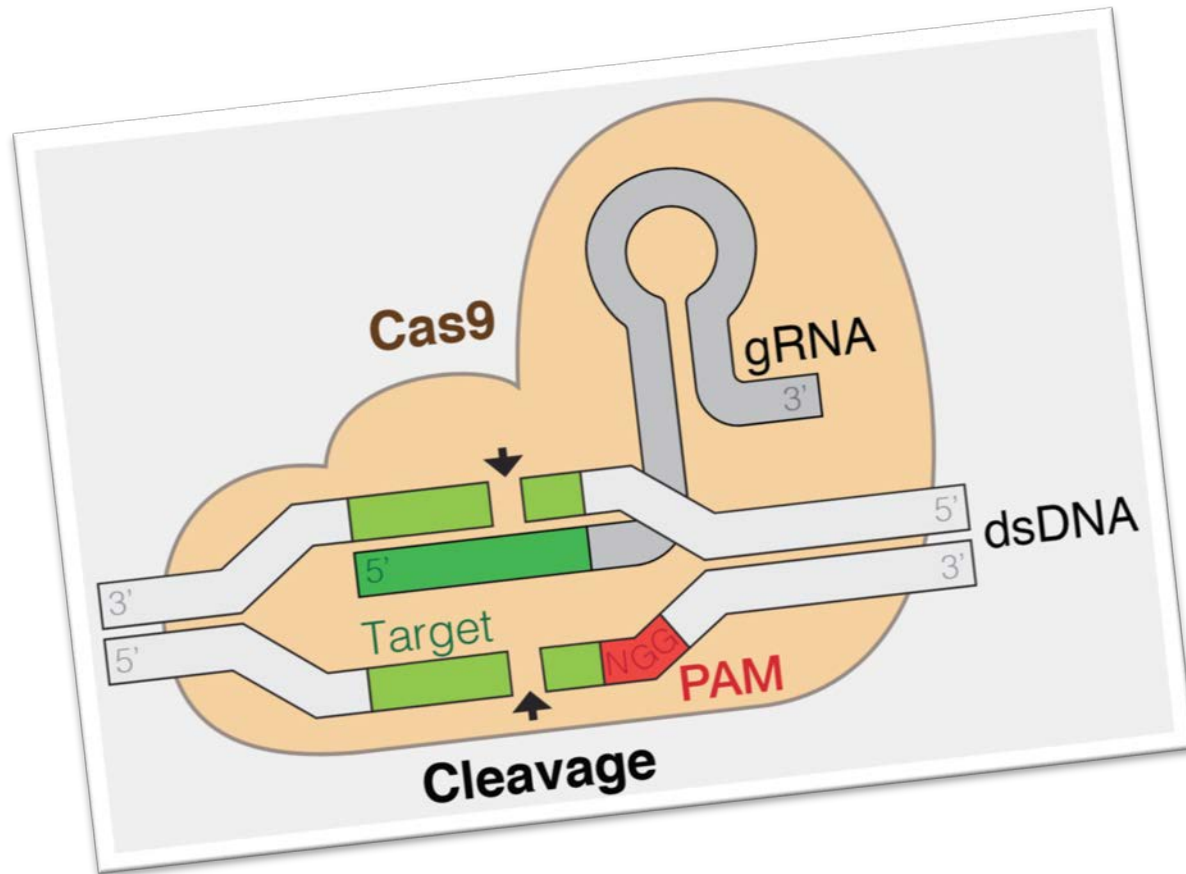
CRISPR



Title	Phase I Trial of Autologous T Cells Engineered to Express NY-ESO-1 TCR And Gene Edited to Eliminate Endogenous TCR and PD-1
Purpose	To determine the safety and feasibility of administering NYCE cells in adult cancer patients.
How?	CRISPR guided RNA is added to T cells to disrupt expression of certain proteins and antibodies.

STUDIES REVIEWED & NOW AT PENN:

CRISPR



How does CRISPR work?

- Harnessed from bacterial cell immune systems.
- “Spacer” sequences in CRISPR guide the system to matching sequences of DNA. Cas9 binds to the DNA and cuts it, shutting the targeted gene off.
- Genes can be permanently modified.

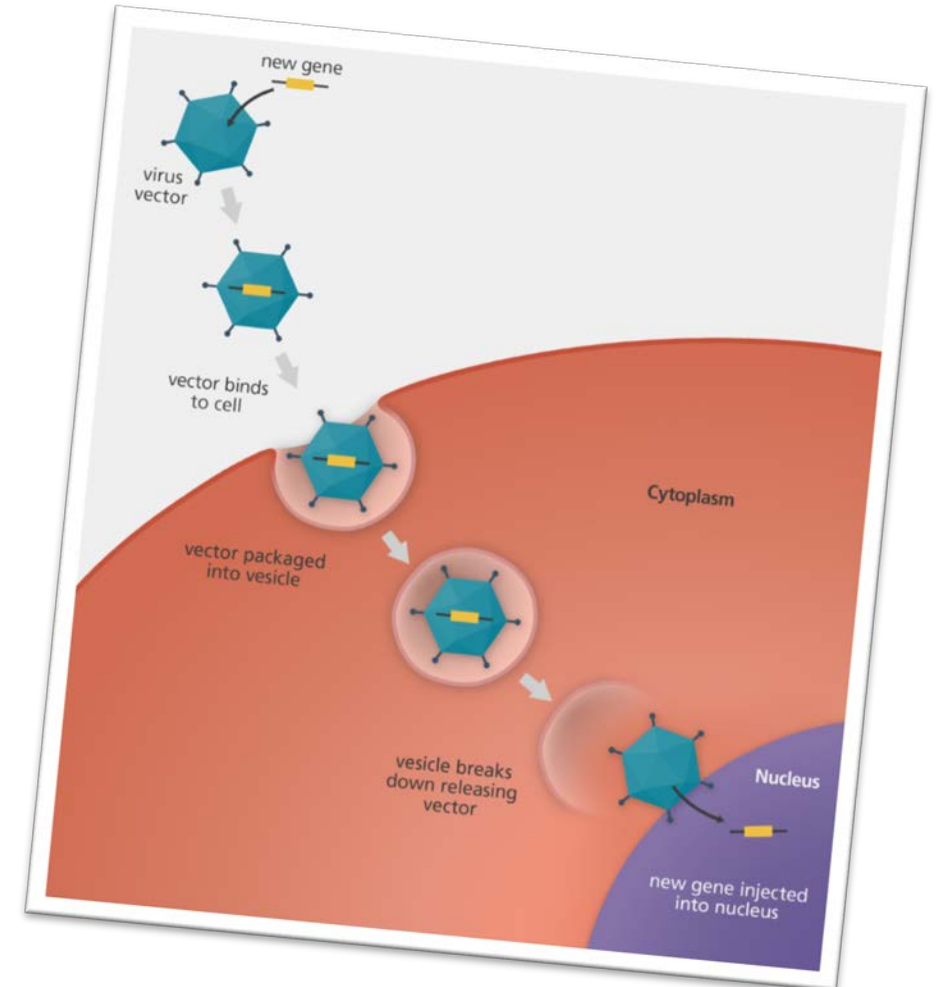
Benefits

- Very accurate, very customizable, and relatively inexpensive
- Can also target many genes at once

STUDIES REVIEWED & NOW AT PENN:

GENE THERAPY for MPS I

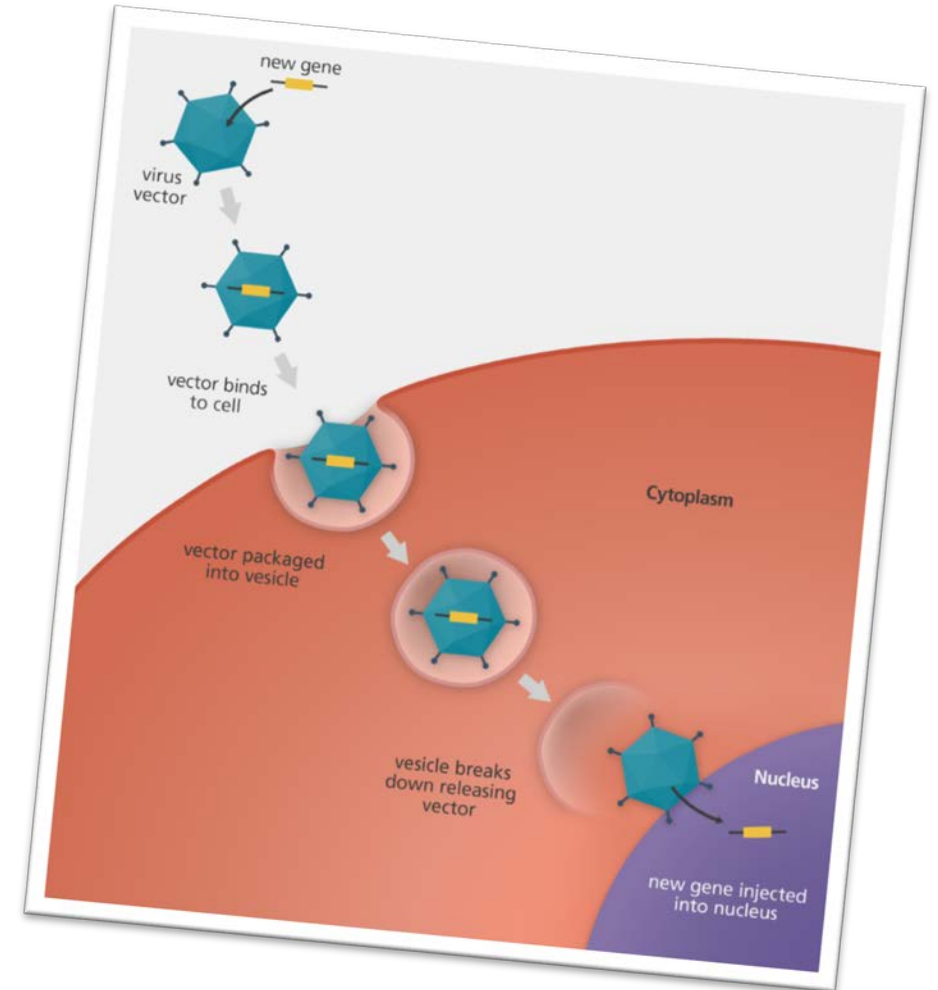
Title	Phase I Multicenter, Open-Label Study to Evaluate the Safety, Tolerability, and Pharmacodynamics of Intracisternal RGX-111 Gene Therapy in Subjects with Mucopolysaccharidosis Type I
What is MPS I?	<p>MPS I is an inherited, genetic disorder. Long chains of sugar molecules build up, cause damage to cells, and stop normal cell function.</p> <p>Current Clinical State: Subjects may be 6 years and older. Standard of care treatment options are temporary and only have low levels of effectiveness.</p>



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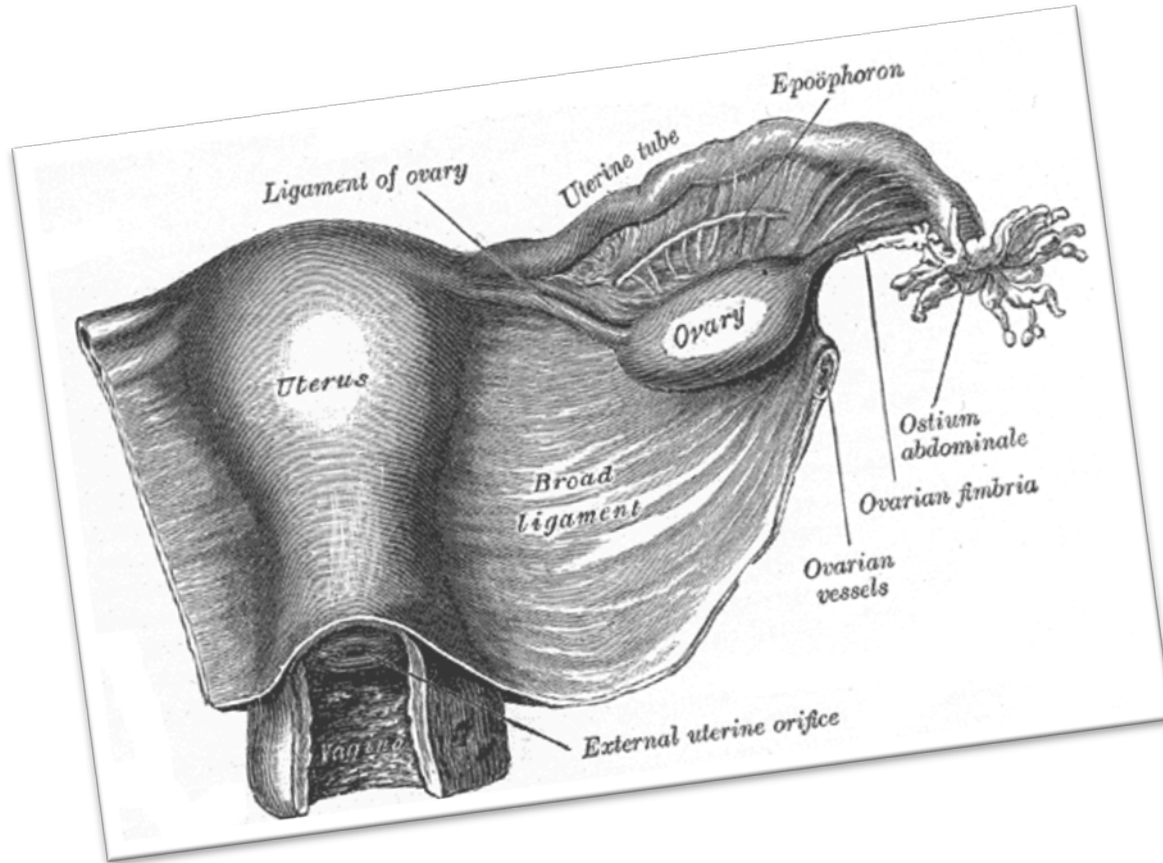
GENE THERAPY for MPS I

What is RGX 111?	Product contains a working gene for the IDUA enzyme
How does it work?	<p>Will be given by inserting a needle into the back of the head below the brain. CT scan images will be used to guide the insertion.</p> <p>Purpose of Study: to cross the blood-brain barrier, which is impossible with current standard care treatments.</p>



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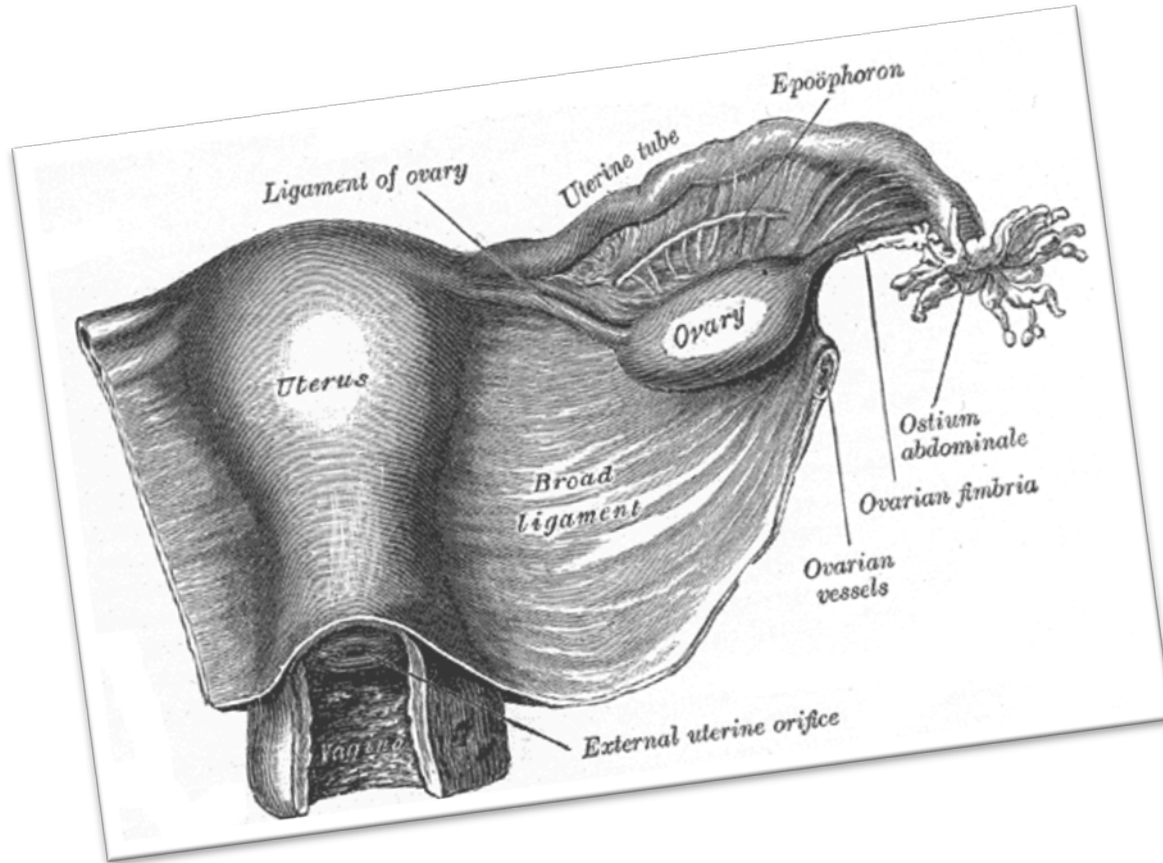
UTERINE TRANSPLANT



Title	Penn UteriNe Transplantation for uterine factor Infertility (UNTIL) Trial
Purpose	<p>Evaluate the feasibility of uterine transplantation using a deceased donor uterine transplantation model to treat absolute uterine factor infertility (AUI)</p> <p>Primary Objective: <i>achieve a successful engraftment of deceased donor uterine transplant</i></p>

STUDIES REVIEWED & NOW AT PENN:

UTERINE TRANSPLANT



Target Population

Penn UteriNe Transplantation for uterine factor Infertility (UNTIL) Trial

Procedures

Multi-disciplinary team of physicians, nurses, social workers, nutritionists and pharmacists actively participating

An immunosuppression management plan has been incorporated in the protocol that is modeled after the Swedish group that was able to achieve the only human experience of live birth from deceased donor uterine transplant, to date

METHOD: Not only were the Boards **innovative** in what and how much they reviewed, but also **in the way reviews happen** to maintain the place on the frontier of ethical review.

**System Changes
to Quicken
Review**

- Addition of IRB-specific forms, which are continually updated per changes in regulations
- Incorporation of PDF portfolios & new .zip files

**Direct Education
for Study Teams**

- Began **direct trainings** for study team who demonstrate additional need
- Implementation of **more beginner sessions** for new coordinators & students

**More Ways to
Facilitate
Meeting Review**

- Boards reflected & implemented ways to manage reviews, including **tackling minimalist submissions**



THANK YOU FOR YOUR
EXCEPTIONAL WORK!

We really appreciate all you do,
for the IRB, for Penn, and for
research as a whole, and we
couldn't do it without you!