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.
**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.
### 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

<table>
<thead>
<tr>
<th>Title</th>
<th>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</th>
</tr>
</thead>
<tbody>
<tr>
<td>What is MPS I?</td>
<td>MPS I is an inherited, genetic disorder. Long chains of sugar molecules build up, cause damage to cells, and stop normal cell function.</td>
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</table>
**STUDIES REVIEWED & NOW AT PENN:**

**GENE THERAPY for MPS I**

<table>
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<tr>
<th>What is RGX 111?</th>
<th>Product contains a working gene for the IDUA enzyme</th>
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</table>
| How does it work? | 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.  
**Purpose of Study:** to cross the blood-brain barrier, which is impossible with current standard care treatments. |
STUDIES REVIEWED & NOW AT PENN: UTERINE TRANSPLANT

Title | Penn UteriNe Transplantation for uterine factor InfertiLity (UNTIL) Trial

Purpose | Evaluate the feasibility of uterine transplantation using a deceased donor uterine transplantation model to treat absolute uterine factor infertility (AUFI)

Primary Objective: achieve a successful engraftment of deceased donor uterine transplant
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!