

## TRANSPLANT THERAPEUTICS CONSORTIUM (TTC)



In 2017, the American Society of Transplant Surgeons and the American Society of Transplantation partnered with the Critical Path Institute to create the <u>Transplant Therapeutics Consortium</u>.

#### TTC is a public-private partnership

among scientists from the biopharmaceutical industry, diagnostics companies, academic institutions, professional societies and their patient advocacy groups, and government and regulatory agencies.

As the only community-based group, TTC is dedicated to **advancing the regulatory science needs of transplant.** 

## **OUR MISSION**

Our primary effort is FDA qualification of the **iBOX as a reasonably likely surrogate endpoint (RLSE)** for long-term graft survival after kidney transplantation.

iBOX is the only endpoint in FDA Biomarker Qualification Program addressing patient, regulatory, and clinician needs.

**Qualified biomarkers** and regulatoryendorsed tools are made **publicly available** to benefit the community and to improve future clinical trial efficiency.

## **HOW WE DO IT**

TTC identifies challenges, prioritizes solutions, and **develops tools** to advance new product development to meet the most pressing needs of transplant recipients.

TRANSPLANT THERAPEUTICS

CONSORTIUM CRITICAL PATH INSTITUTE

TTC has the **largest kidney transplant data repository** of individual clinical trials and real-world datasets to date to support the FDA qualification submission of iBOX as a RLSE.

We are deeply aware that data can offer key insights to guide and improve the development of new therapies in transplantation.

# <u>Primary effort</u>: To qualify iBOX as a RLSE for long-term graft survival after kidney transplantation

- iBOX, <u>Loupy et al., 2019</u>, led by the Paris Transplant Group, is the best surrogate for late graft failure after kidney transplantation
- Extensive epidemiologic and prognostic data (n = 4,000)
- Strong mechanistic data for each component
- Comprehensive assessment of kidney graft health
- 2 iBOX versions:
  - Full (with biopsy)
  - Abbreviated (without biopsy)

## **KEY FOCUS AREAS**

Promote development of new regulatory approaches supporting innovative research with patient-focused drug development at the core of all work streams



Foster awareness of the importance of data sharing to support TTC initiatives

Establish consensus among stakeholders in preferred tools to accelerate drug development for transplant therapeutics



nuria Biopsy <u>findings</u>

4. Banff Lesion

#### iBOX meets ALL criteria for FDA Accelerated Approval

✓ Treats a serious condition
● Graft loss

• Graft loss

✓ Provides a meaningful advantage over available therapies

- Allows superiority of a new therapy and a new indication
- ✓ Demonstrates an effect on a <u>surrogate endpoint</u> <u>that is reasonably likely</u> to predict clinical benefit or on a clinical endpoint that can be <u>measured</u> <u>earlier</u> than irreversible mortality or morbidity

 iBOX as a RLSE at 1 year for 5-year graft survival

## **OUR SUCCESSES AND MILESTONES**



First qualified endpoint for kidney transplantation by regulatory agency. TTC received a <u>Qualification Opinion</u> by EMA for the <u>iBOX</u> as a secondary efficacy endpoint in kidney transplant clinical trials.



Built a publicly available <u>Sample size calculator using iBOX scores</u> to assist with trial design.

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Conducted a <u>study</u> that successfully used UNOS registry data as external controls to supplement internal controls for long-term survival in Accelerated Approval

## **CONSORTIUM MEMBERS**

TTC is supported by funds from the transplant community, including the biopharmaceutical and diagnostic industries, professional societies, and regulatory agencies, combined with support from academic institutions, ensuring that people living with a transplant are at the core of all we do.

#### **FOUNDING PARTNERS**

- American Society of Transplantation
- American Society of Transplant Surgeons
- Critical Path Institute

## GOVERNMENT AND REGULATORY AGENCIES

- National Institute of Health
- U.S. Food and Drug Administration

## **PROFESSIONAL SOCIETIES**

- American Societies of Transplantation
- American Society of Transplant Surgeons
- The Transplantation Society



#### **BIO-PHARMACEUTICAL INDUSTRY**

- Argenx
- Arkana Laboratories
- Bristol-Myers Squibb
- CareDx
- CSL Behring
- Eledon Pharmaceuticals
- Eurofins Transplant Genomics
- Hansa Biopharma
- Immucor
- Pirche
- Sanofi
- Takeda
- Veloxis

#### ACADEMIC INSTITUTIONS

- Charité-Berlin
- Helsinki University Hospital
- Hospital do Rim
- Houston Methodist Hospital
- Katholieke Universiteit te Leuven
- Mayo Clinic Rochester
- Necker Hospital, Paris Transplant Group
- University of British Columbia
- University of Colorado, Denver
- University of Wisconsin, School of Medicine

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CRITICAL PATH INSTITUTE



TTC Website



**TTC Publications** 

Founded in 2005, Critical Path Institute was formed as a nonprofit organization to serve as a neutral third party to facilitate dynamic worldwide collaboration among scientists. C-Path was established with broad-based support from the Tucson community, government, and Science Foundation Arizona.