



UNITED THERAPEUTICS CORPORATION

Jefferies 2017 Global Healthcare Conference New York City

Safe Harbor Statement and Non-GAAP Information

Remarks today concerning

United Therapeutics may include forward-looking statements which represent United Therapeutics' expectations or beliefs regarding future events. We caution that such statements involve risks and uncertainties that may cause actual results to differ materially from those in the forward-looking statements. Consequently, all such forward-looking statements are qualified by the cautionary language and risk factors set forth in United Therapeutics' periodic and other reports filed with the SEC.

There can be no assurance

that the actual results, events or developments referenced in such forward-looking statements will occur or be realized. United Therapeutics assumes no obligation to update these forward-looking statements to reflect actual results, changes in assumptions or changes in factors affecting such forward-looking statements.

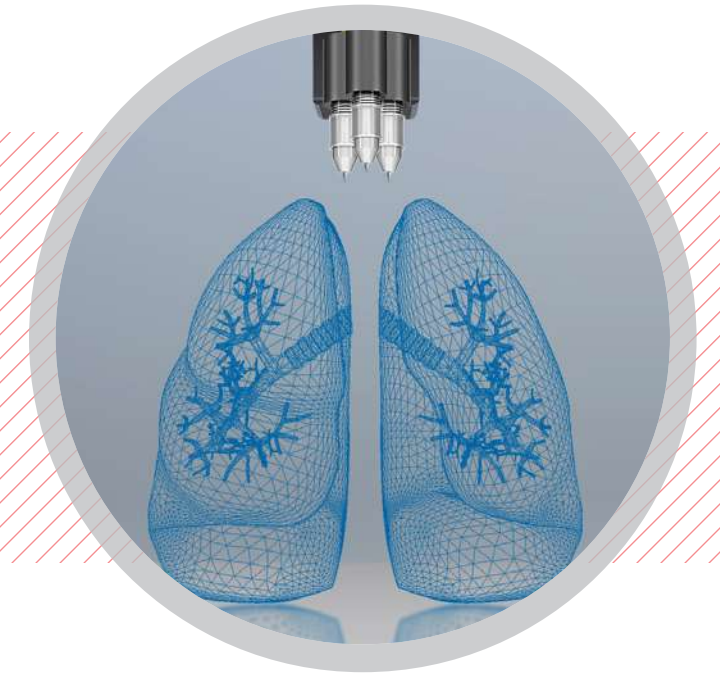
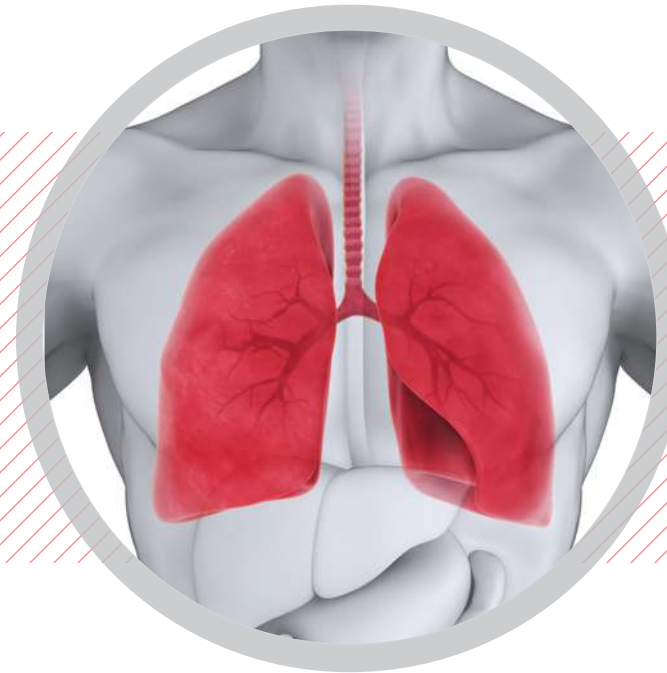
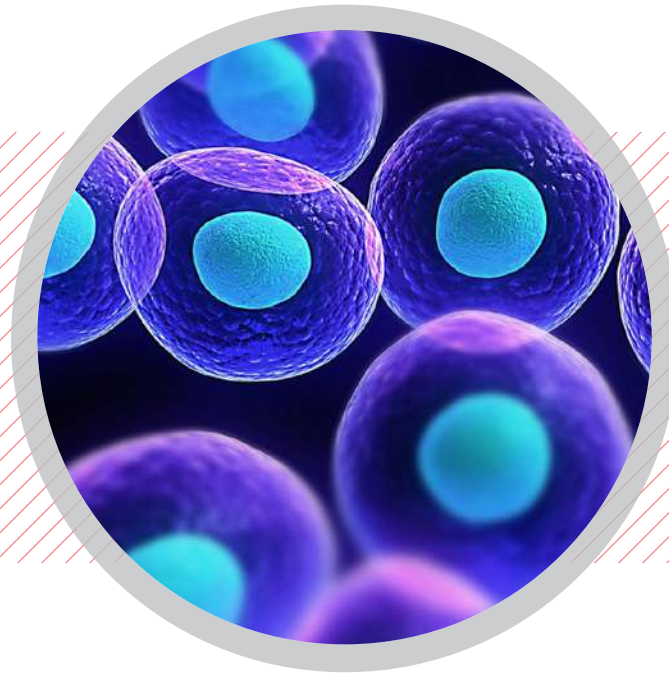
The discussions

during this presentation could include certain financial measures that were not prepared in accordance with U.S. Generally Accepted Accounting Principles (GAAP). Reconciliations of those non-GAAP financial measures to the most directly comparable U.S. GAAP financial measures can be found in our earnings releases filed with the SEC in Current Reports on Form 8-K for the relevant time period. These reports are available on our website at www.unither.com in the "Investor Relations Financial Information SEC Filings" section.

This presentation

and any related discussions or statements are intended to educate investors about our company. Sometimes that process includes reporting on the progress and results of clinical trials or other developments with respect to our products. This presentation and any related discussions or statements are not intended to promote our products, to suggest that our products are safe and effective for any use other than what is consistent with their FDA-approved labeling, or to provide all available information regarding the products, their risks, or related clinical trial results. Anyone seeking information regarding the use of one of our products should consult the full prescribing information for the product available on our website at www.unither.com.

UT FACTs



Focus

Advance

Corridor

Transform

▶ **FOCUSED** on Patients to Deliver Long-term Revenue Growth

Treating more PAH patients in the US than any other company

2016

\$1.6B
in Revenues⁽¹⁾

>\$600M
in free cash flow


orenitram[®]
treprostini
EXTENDED-RELEASE TABLETS


REMODULIN[®]
(treprostini) Injection

TYVASO[®]
(treprostini) **INHALATION**
SOLUTION


adcirca[®]
tadalafil tablets 20mg


Unituxin[®]
(dinutuximab)
Injection

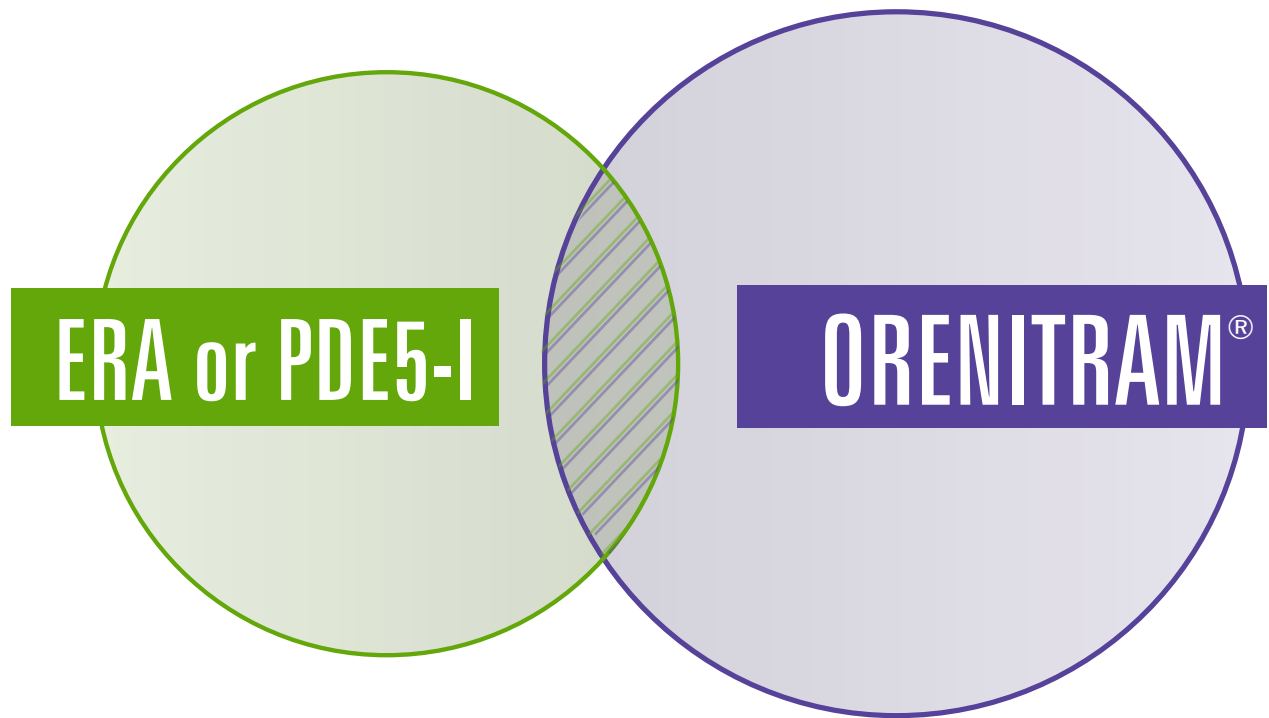
(1) As reported in the annual Form 10-K for the period ended December 31, 2016.

▶ **ADVANCE** Improved Therapeutic Benefit with Our **FREEDOM-EV** Combination Therapy Clinical Trial

ORENIPLUS™⁽¹⁾
for PAH

Extending life
by decreasing
morbidity/mortality

FREEDOM-EV
Combination Therapy



(1) OreniPlus refers to our effort to expand Orenitram's label to indicate that Orenitram delays morbidity and/or mortality in PAH patients who are on an approved oral background therapy.

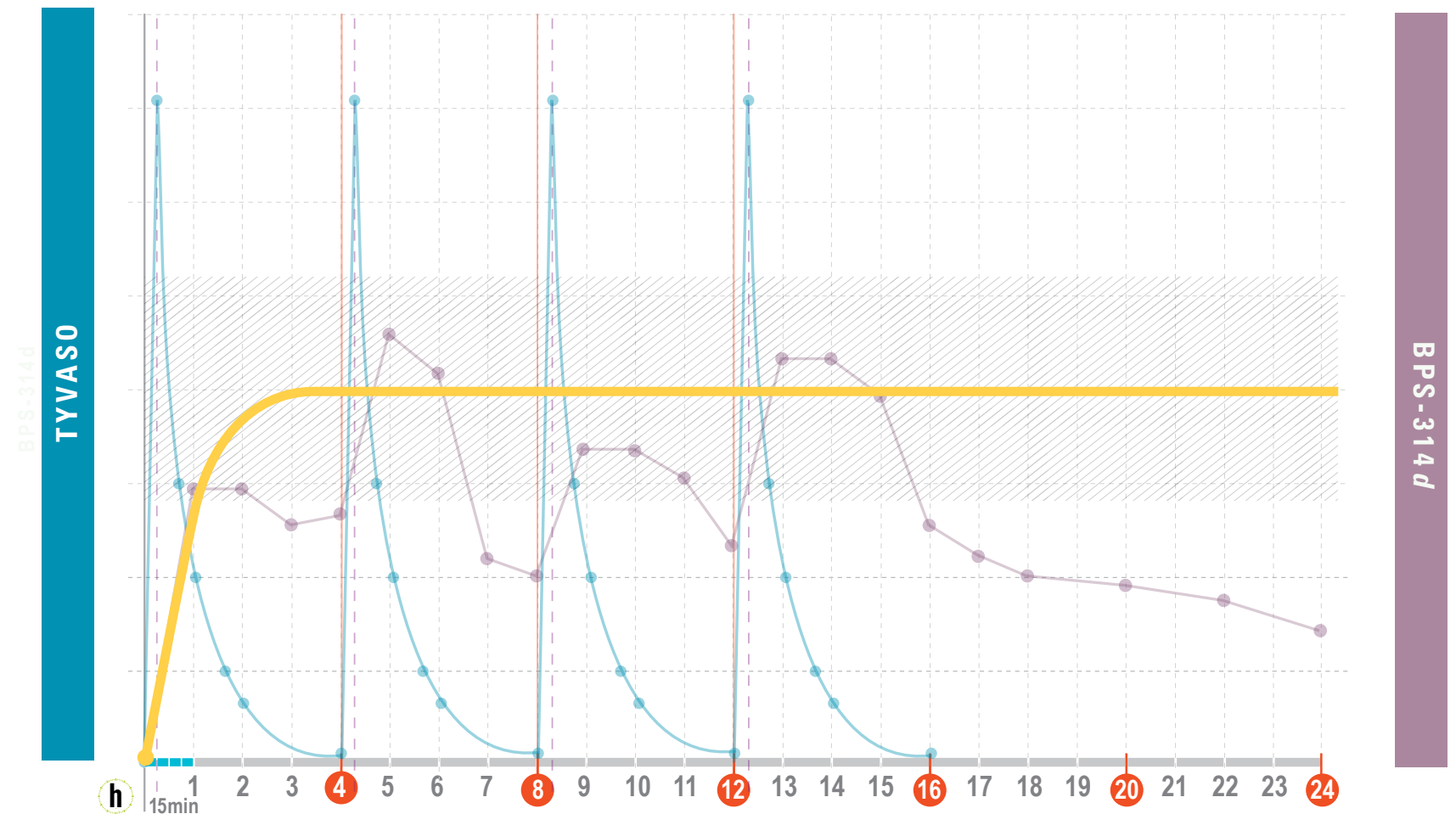
ADVANCE Improved Therapeutic Benefit with Our BEAT Combination Therapy Clinical Trial

TYSUBERPROST™⁽¹⁾
for PAH

Extending life
by decreasing
morbidity/mortality

(1) Tysuberprost refers to the combination use of esuberaprost (which has not been approved by FDA) and Tyvaso®.

BEAT (BERAPROST 314d ADD-ON TO TYVASO)

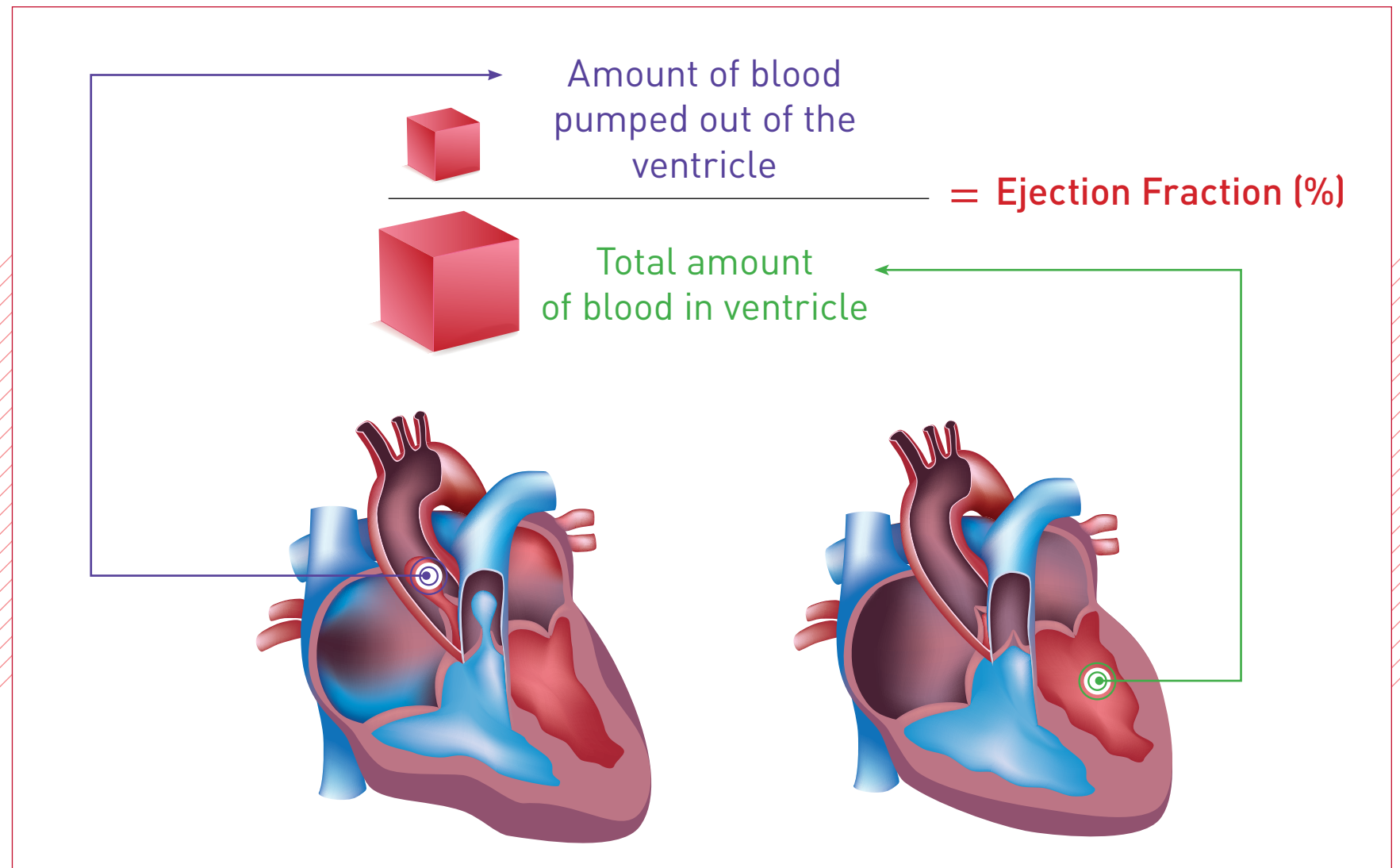


“CORRIDOR of Indifference” in PH WHO Group 2 with Our SOUTHPAW Clinical Trial

ORENILEFT™⁽¹⁾
for PH HFpEF⁽²⁾

>50,000
PATIENTS

(1) Orenitram is not FDA approved for Who Group 2 PH patients.
(2) PH HFpEF=Pulmonary Hypertension associated with Heart Failure with Preserved Ejection Fraction.



“CORRIDOR of Indifference” in PH WHO Group 3 with Our INCREASE Clinical Trial

TYVASO-ILD™⁽¹⁾
for PH ILD⁽²⁾

>30,000
PATIENTS

(1) Tyvaso is not FDA approved for Who Group 3 PH patients.
(2) PH ILD = Pulmonary Hypertension associated with Interstitial Lung Disease.



Interstitial Lung Disease

▶ **TRANSFORM** the Marketplace to Make **More Organs** Available for Transplant

~**1.0** Million

people in the US that have end-stage organ disease and may need a heart, kidney or lung transplant

- ▶ EX-Vivo Lung Perfusion (EVLP)
- ▶ Xenotransplantation
- ▶ Engineered and Personalized Lungs

▶ **TRANSFORM** “Science Fiction” into “Science Fact” **with EVLP**

To date, EVLP technology has **successfully transplanted**

>500
PATIENTS⁽¹⁾

(1) Global figure including all EVLP technologies.



XENOTRANSPLANTATION Transforming the Marketplace to Make More Organs Available for Transplant

Porcine Xenotransplantation milestones in baboons

Kidney graft ▶ **> 8 months**


Heart graft ▶ **> 2 years**

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ORIGINAL ARTICLE

WILEY Xenotransplantation

Immunological and physiological observations in baboons with life-supporting genetically engineered pig kidney grafts

Hayato Iwase¹ | Hidetaka Hara¹ | Mohamed Ezzelarab¹ | Tao Li^{1,2} |
Zhongqiang Zhang^{1,3} | Bingsi Gao^{1,2} | Hong Liu^{1,4} | Cassandra Long¹ |
Yi Wang² | Amy Cassano⁵ | Edwin Klein⁵ | Carol Phelps⁶ | David Ayares⁶ |
Abhinav Humar¹ | Martin Wijkstrom¹ | David K. C. Cooper¹ 

¹Thomas E. Starzl Transplantation Institute, University of Pittsburgh, Pittsburgh, PA, USA

²Center for Kidney Transplantation, Second Affiliated Hospital of the University of South China, Hengyang, Hunan, China

³Department of General Surgery, Second Xiangya Hospital of the Central South University, Changsha, Hunan, China

⁴Department of General Surgery, First Hospital of Shanxi Medical University, Taiyuan, China

⁵Division of Laboratory Animal Resources, University of Pittsburgh, Pittsburgh, PA, USA

⁶Revivicor, Blacksburg, VA, USA

Correspondence
David K. C. Cooper, Thomas E. Starzl Transplantation Institute, University of Pittsburgh Medical Center, Pittsburgh, PA, USA.
Email: cooperdk@upmc.edu

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Abstract

Background: Genetically engineered pigs could provide a source of kidneys for clinical transplantation. The two longest kidney graft survivals reported to date have been 136 and 310 days, but graft survival >30 days has been unusual until recently.

Methods: Donor pigs (n=4) were on an α 1,3-galactosyltransferase gene-knockout (GTKO)/human complement regulatory protein (CD46) background (GTKO/CD46). In addition, the pigs were transgenic for at least one human coagulation regulatory protein. Two baboons received a kidney from a six-gene pig (GroupA) and two from a three-gene pig (GroupB). Immunosuppressive therapy was identical in all four cases and consisted of anti-thymoglobulin (ATG)+anti-CD20mAb (induction) and anti-CD40 mAb+rapamycin+corticosteroids (maintenance). Anti-TNF- α and anti-IL-6R mAbs were administered to reduce the inflammatory response. Baboons were followed by clinical/laboratory monitoring of immune/coagulation/inflammatory/physiological parameters. At biopsy or euthanasia, the grafts were examined by microscopy.

Results: The two GroupA baboons remained healthy with normal renal function >7 and >8 months, respectively, but then developed infectious complications. However, no features of a consumptive coagulopathy, eg. thrombocytopenia and reduction of fibrinogen, or of a protein-losing nephropathy were observed. There was no evidence of an elicited anti-pig antibody response, and histology of biopsies taken at approximately 4, 6, and 7 months and at necropsy showed no significant abnormalities. In contrast, both GroupB baboons developed features of a consumptive coagulopathy and required euthanasia on day 12.

Conclusions: The combination of (i) a graft from a specific six-gene genetically modified pig, (ii) an effective immunosuppressive regimen, and (iii) anti-inflammatory therapy prevented immune injury, a protein-losing nephropathy, and coagulation dysfunction for >7 months. Although the number of experiments is very limited, our impression is that expression of human endothelial protein C receptor (\pm CD55) in the graft is important if coagulation dysregulation is to be avoided.

▶ **TRANSFORM** Transplant Rejection with Engineered and Personalized Lungs

Lungs built out of a patient's own lung cells, thus **reducing or eliminating the risk of:**

- ▶ Transplant rejection
- ▶ Immunosuppressant therapies



Decellularized Lung Scaffolds

**UNITED
THERAPEUTICS**
**ONE OF THE
MOST IMPORTANT
COMPANIES
FOR HUMAN
LONGEVITY**

