

# *Public-Private Partnerships: New models for Collaborative Biomedical Research at UCSF*

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Executive Vice Chancellor and Provost, UCSF  
August 2013*

**UCSF**

University of California  
San Francisco



# *The Inconvenient Truth*

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The number of approved first-in-class approved drugs for the treatment/cure of diseases is abysmal

Government, Industry and Academia partnerships to develop disease treatments cures have largely failed

U.S. venture capitalists are decreasing their investment in biotechnology and medical device start-ups, their concentration in critical therapeutic areas, and shifting focus away from the United States

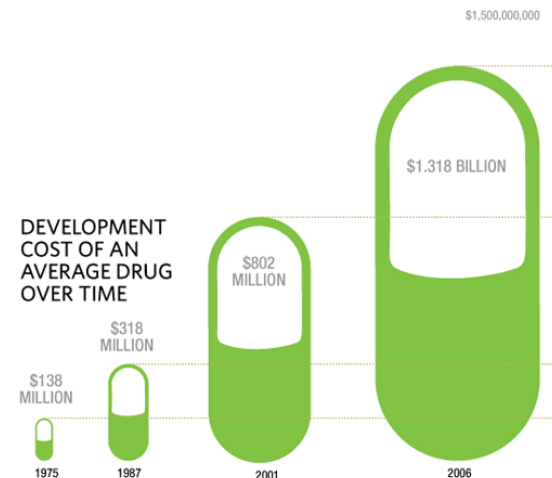
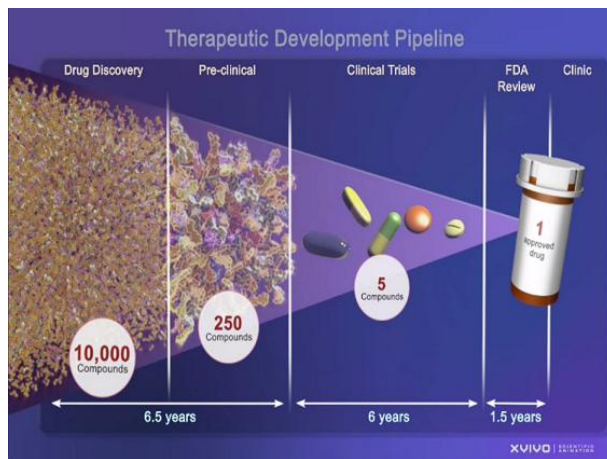
The failure rates, cost, regulatory hurdles, difficulty in patient recruitment and lack of public support of drug development have reached all time highs.



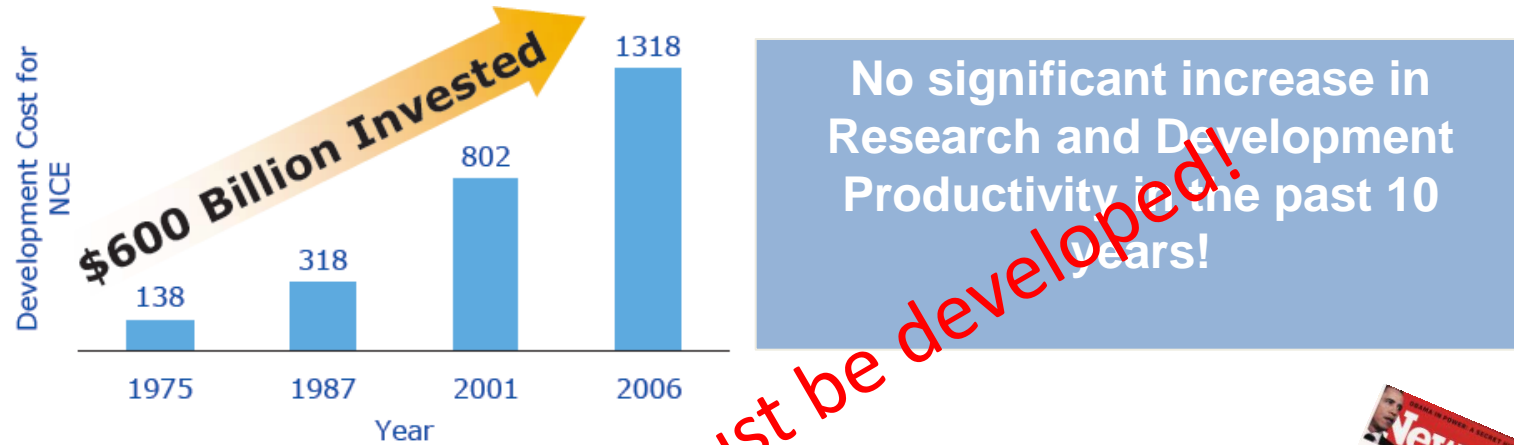
# *The Research and Development process is problematic*

## Each New Drug

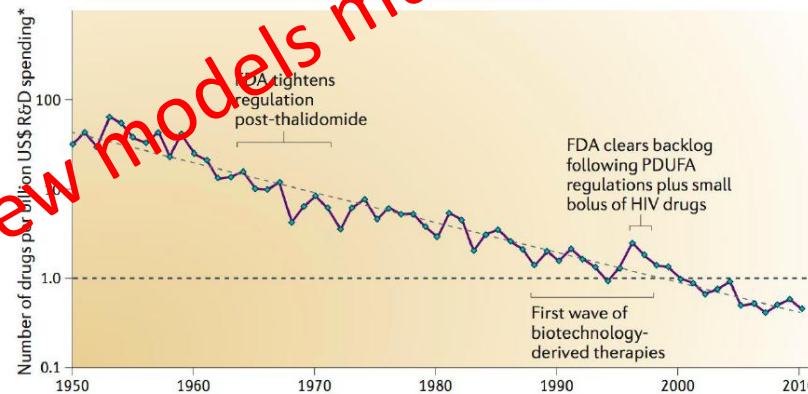
- Takes more than 10 years to develop
- Requires an investment of over \$1B to bring a single innovative drug to market
- Years in the regulatory path
- No guarantee of insurance reimbursement



# Number of new drugs approved has plateaued over the past 10 years



**New models must be developed!**



**FIGURE 2-3** The number of new drugs approved per billion dollars spent has declined steadily on a logarithmic scale for more than a half century.  
SOURCE: Scannell et al., 2012.

# Pressures are increasing to develop new and innovative Industry-Academic Partnerships

BIOTECH, DRUGS, STARTUPS

## The Pharmaceutical R&D Model is Broken. Here's How to Fix It

Stewart Lyman 3/5/10

Research is the lifeblood of the biotech and pharmaceuticals business. The pharma and biotech industry spent some \$65 billion dollars on R & D in 2008, according to the Pharmaceutical Research and Manufacturers Association. That's a tremendous amount of money considering that the FDA of molecular entities and 3 bio

*Clinical Pharmacology & Therapeutics* **87**, 525-527 (May 2010) | doi:10.1

## The Future Is Much Closer Collaboration Between the Pharmaceutical Industry and Academic Medical Centers

P Vallance, P Williams and C Dollery

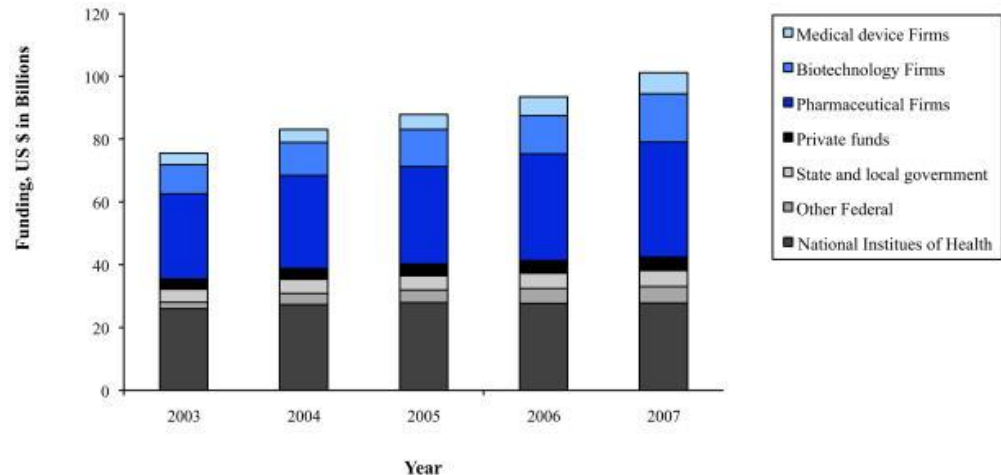
A reader of newspaper articles and editorials in some medical

GlaxoSmithKline: enhancing R&D with greater academic expertise.

4th of March 2011 @ 11:27 | Source: Bioportfolio

Recommend Tweet 2 +1 0

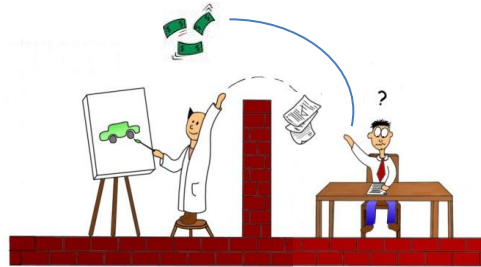
Home > Glaxosmithkline Channel > Article > GlaxoSmithKline: enhancing R&D with greater academic expertise.



While NIH funding has been relatively flat, funding from private industry sources has increased over the past few years.



# However.....



## *Academia/Industry/Government partnerships have historically been mainly exchange models*

- Many industry-academia interactions have not been collaborative
- Sponsored research - typically funds idea with first option to license any resulting IP - NIH has historically been largely R01 focused
- Licenses, start-ups reflect taking up idea after conception
- Exchanges are indirect, with limited joint intellectual engagement

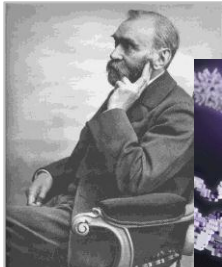
# Historical Challenges to Industry-Academic Alliances

## Differing Incentives, Goals, Cultures

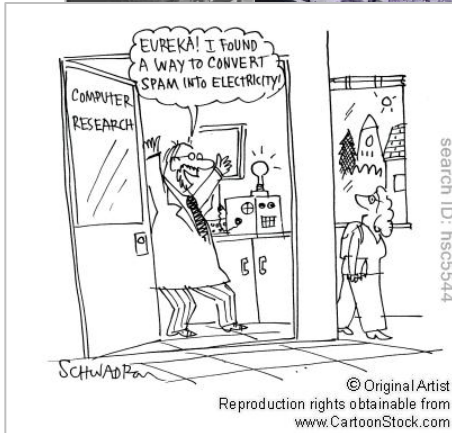
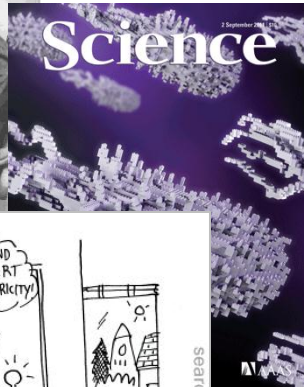
### Academia

### Industry

Recognition

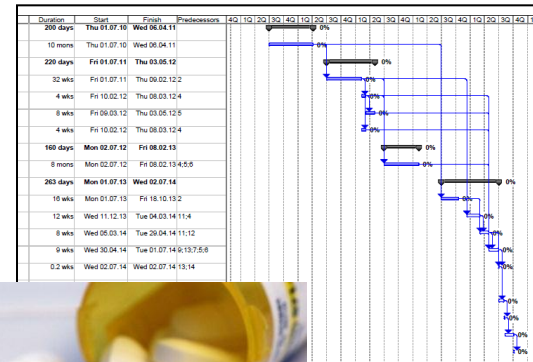


Publications



Scientific freedom

Timeline/Milestones



Products



Exclusivity

# *The New Public-Private Partnership Model: Mutual Investment/ Mutual Benefits*

## *UCSF*

- Enhances faculty research in translational science
- Educational opportunities for students/post-docs
- Access to tools and capabilities not readily available in academia

## *Company*

- Early access to new targets and biology, accelerating translation of targets into therapies
- Enhancement of research and clinical trials via close involvement of experts in mechanistic and therapeutic areas

*Catalyze discovery and development by leveraging the combined capabilities*

*Bring new therapies to patients – meeting AMC, NIH and stockholder missions*



# Building an effective PPP at UCSF

*The Mission Bay  
Innovative Ecosystem*

UCSF-based innovation ecosystem:  
to drive innovation in the Bay Area and globally

Novel Industry partnerships: to accelerate biologic drug  
development



Precision Medicine: Mechanism, not disease based,  
university and industry-based partnerships to advance  
clinical research



# Mission Bay - building a new center of innovation Circa - 2000



# Ecosystem Community – Mission Bay:

## Marrying research, clinical care, and industry

2012

Arch Ventures  
 Column Group  
 Novo Ventures  
 Synergenics  
 USVP  
 Versant Ventures

- Abunda
- Ablexis
- Allopartis
- Carmot Therapeutics
- CV Ingenuity
- Delpor
- Entrotech
- Gemmus Pharma
- GigaGen
- Green Pacific Biologicals
- Kanjilla
- Kilimnjaro
- Kiverdi
- Locus Development
- Lypro Biosciences, Inc.
- Medicus Biosciences
- Metafold Therapeutics
- MLC Dx
- Nichi Bei Bio
- Omnix
- Oncosynergy
- Osprey Pharmaceuticals
- Pathway Therapeutics
- Pharmajet
- Photoswitch Biosciences
- Refactored Matrials
- SeaChange Pharmaceuticals
- Siluria Technologies
- Silver Creek
- Solidus Biosciences
- Targenics
- Teselagen
- Tunitas Therapeutics
- Unhwa UCSA
- ZoneOne Pharma



GLADSTONE



UCSF Children, Women,  
 and Cancer Hospital



# *UCSF ecosystem to support Start Ups and Links to Industry and Capital*

## **Resources**

- Start-up in a Box
- Garage Incubator Space



## **Funding**

- Bridging the Gap Funds
- Genus Awards/X-prizes



Mission Bay Capital

## **Start-up Expertise**

- Entrepreneurs in Residence
- Academy of Biomedical Entrepreneurs
- Expert Affiliates

## **Industry Investment**

- Program for Breakthrough Biomedical Research
- New Technology Partnerships
- Cross faculty appointments

# *UCSF: creating novel public-private alliances*

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## Pfizer

QB3 alliance – fostering collaboration in early stage research with 4 UC campuses and Pfizer

CTI – accelerating the path from discovery to the clinic: >20 campuses, Pfizer. UCSF as a cornerstone partner



## Sanofi

Funding breakthrough science

Collaborative oncology clinical development

Target discovery in diabetes



## Onyx

Collaborative oncology discovery all stages of research and clinical development



# *Example of a Partnership: Pfizer Centers for Therapeutic Innovation*

## **CTI VISION**

Accelerate the translation of academic innovative discoveries into early stage Proof-of-Mechanism in the clinic – basic concept → Phase I trial

## **CTI STRATEGY**

Co-localization on MB campus and co-partnership , joint teams for rapid translation into the clinic

## **CTI Approach**

Access to Pfizer clinical infrastructure (PD/PK, drug development)

Equal partnership – IP, goals and strategy alignment

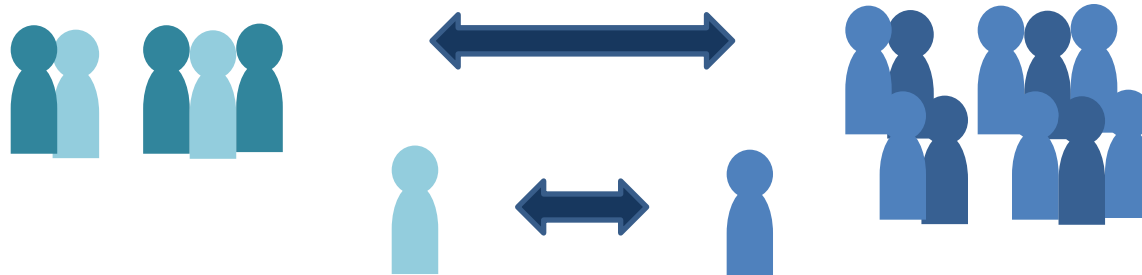


# Joint Development Team Alliance



*UCSF/Sanofi jointly develop and execute strategy to market*

Joint Clinical Development Teams (multiple programs)



## UCSF

### **Clinicians**

Disease treatment expertise

### **Translational Scientists**

Biomarkers animal models  
mechanistic

### **Alliance Management**

## Sanofi-aventis

### **Medical Monitors**

### **Clinical Scientists**

### **Clinical Operations**

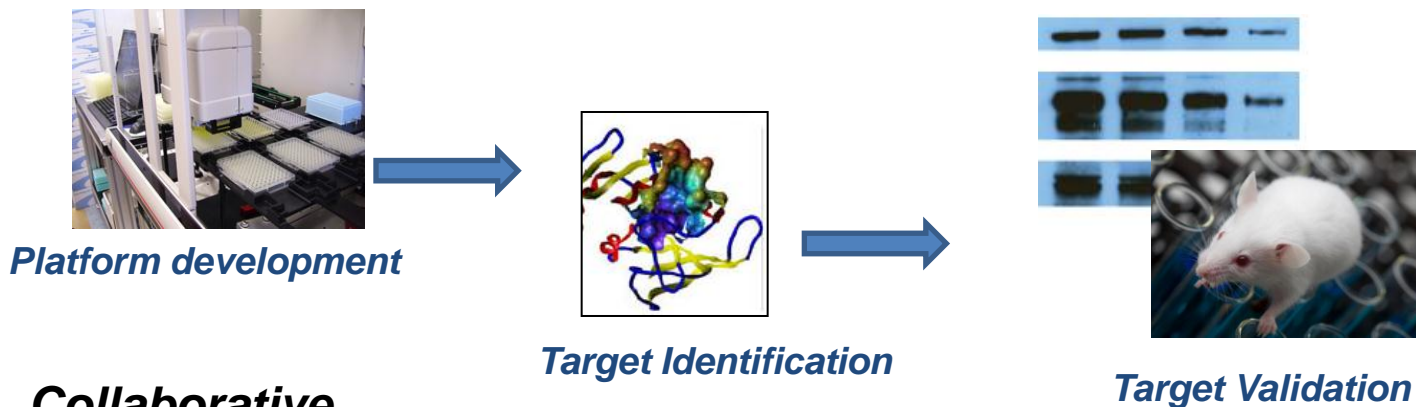
### **Drug production/Packaging**

### **Translational Scientists**

### **Program Management**



# Joint Target Discovery: Alliance Innovation



## **Collaborative**

- Integrated teams with scientists from both academia and pharma

## **Incentives**

- Milestone payments for target deliverables
- Potential for further collaborative drug development or target reverts back to investigator if progression not made by pharma

## **Expectation management/cultural alignment**

- Alliance/program management



# The Immune Tolerance Network:

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AN ACADEMIC – GOVERNMENT – INDUSTRY – FOUNDATION PARTNERSHIP

**NIAID**   
National Institute of  
Allergy & Infectious Diseases

*jointly funded by:*

  
Juvenile Diabetes  
Research  
Foundation  
International  
*dedicated to finding a cure*

Over 40  
industry  
partners

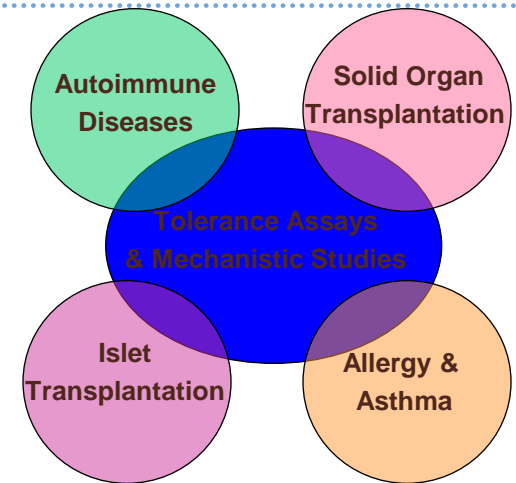
# The Immune Tolerance Network

## What the ITN does

Fund, plan, implement, monitor and assess investigator-initiated clinical trials of novel tolerance-promoting therapies in:

- Autoimmune diseases
- Transplantation
- Allergy & Asthma

Plan and provide services to investigators to carry out unique, comprehensive mechanistic studies



## The ITN Goals

- To advance the clinical application of immune tolerance by performing high quality clinical trials of emerging therapeutics integrated with mechanism-based research.

*In particular, the ITN aims to:*

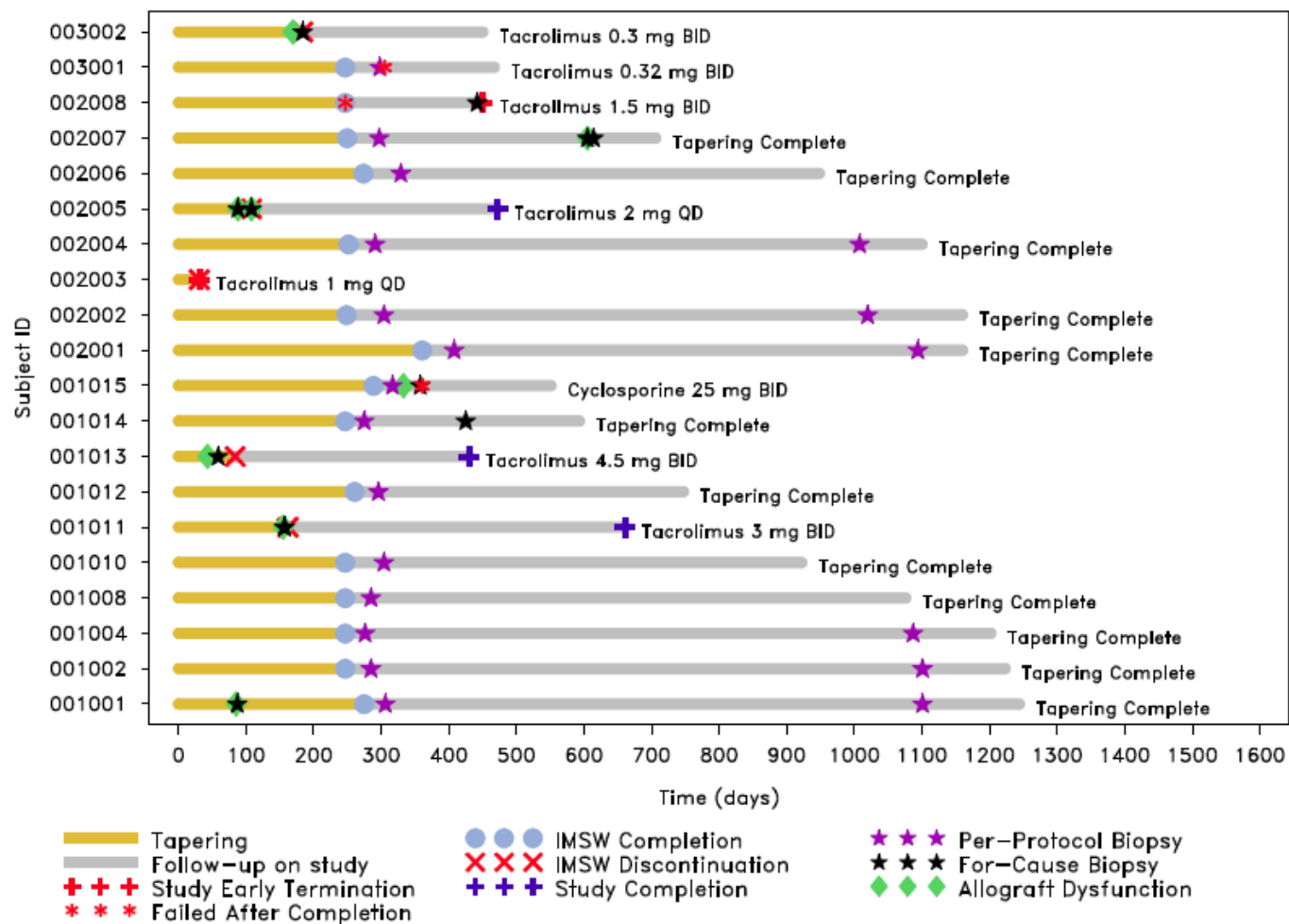
- *establish new tolerance therapeutics*
- *develop a better understanding of the mechanisms of immune function and disease pathogenesis*
- *identify new biomarkers of tolerance and disease.*

## *By the numbers*

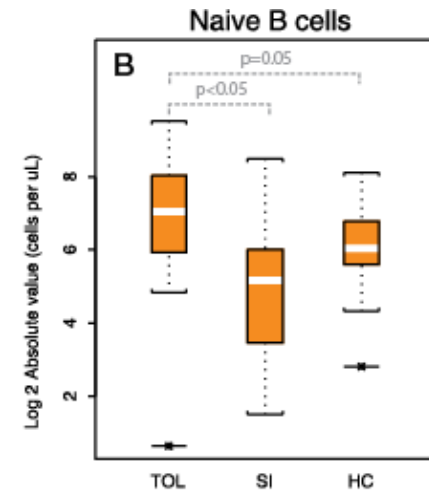
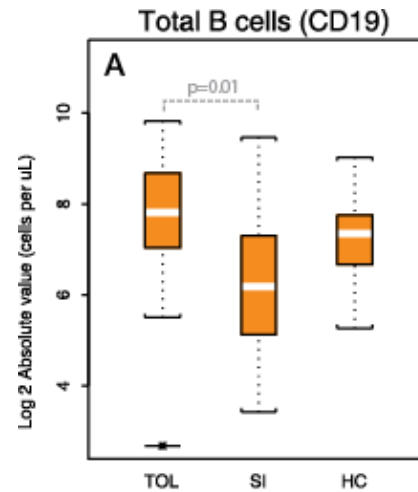
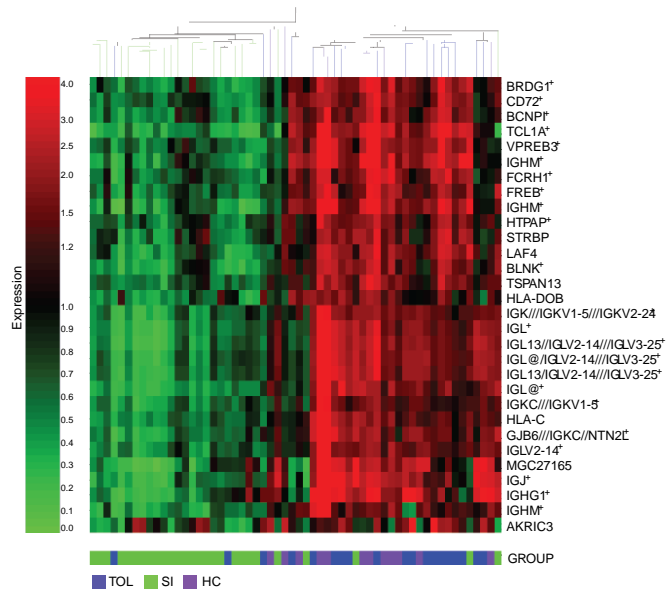
- 10+ countries with ITN sites or members
- ~20 centralized, standardized core assay facilities
- 50+ transplant patients off all immunosuppression
- 30+ clinical trials completed or in progress
- 100+ published manuscripts; 6 NEJM, 150+ meeting presentations
- ~90 full-time employees
- 450+ clinical sites/investigators, around the world
- 6000+ subjects consented in ITN trials
- 25,000+ assays performed by ITN cores
- 400,000+ clinical specimens stored in the ITN repository

*Information-sharing system called TrialShare to instantly access data amassed during the clinical trial*

# Withdrawal of Immunosuppressive drugs in pediatric live-donor livers (Sandy Feng - UCSF)



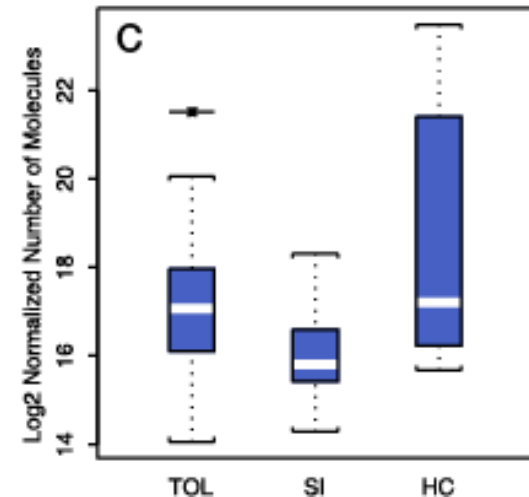
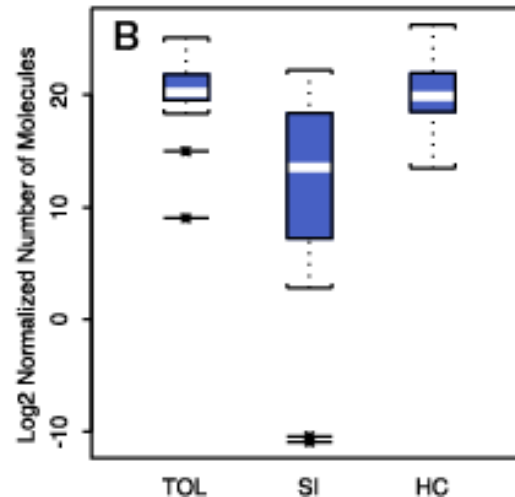
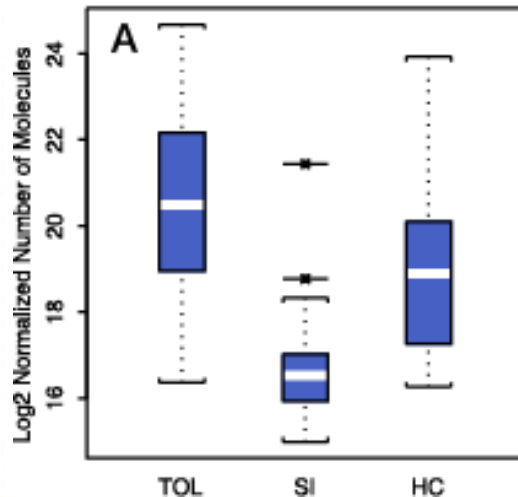
# Increased B cells is a marker of tolerant kidney recipients



IGKV1D-13

IGKV4-1

IGLL1



# Rituximab In ANCA-Associated Vasculitis (RAVE)

PI: **Ulrich Specks**, Mayo Clinic  
**John H. Stone**, Johns Hopkins

In Collaboration with:  
**Genentech**  
IN BUSINESS FOR LIFE

N Engl J Med 2010;363:221-32.

N Engl J Med 2013;369:417-27.

## Study Goals

To determine if B-cell depletion by rituximab induces stable remissions in AAV by re-establishing B-cell tolerance to the ANCA target antigens

## Study Summary

- 194 patient, randomized, double-masked, placebo-controlled trial
- Significantly more patients in the rituximab group reached and retain total remission in the control group even at 18 months ( $P < 0.001$ ).
- The treatment response to rituximab was superior to cyclophosphamide in patients who entered the trial with a severe disease flare ( $P = 0.01$ ).
- Fewer patients in rituximab arm had one or more of the protocol-selected AEs by 6 months.
- Approved new labeling by FDA in 2011.

# Acknowledgements

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QB3 Director of Industry  
Alliance**



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