

Clinical Trial Trends Among Venture Philanthropy Organizations

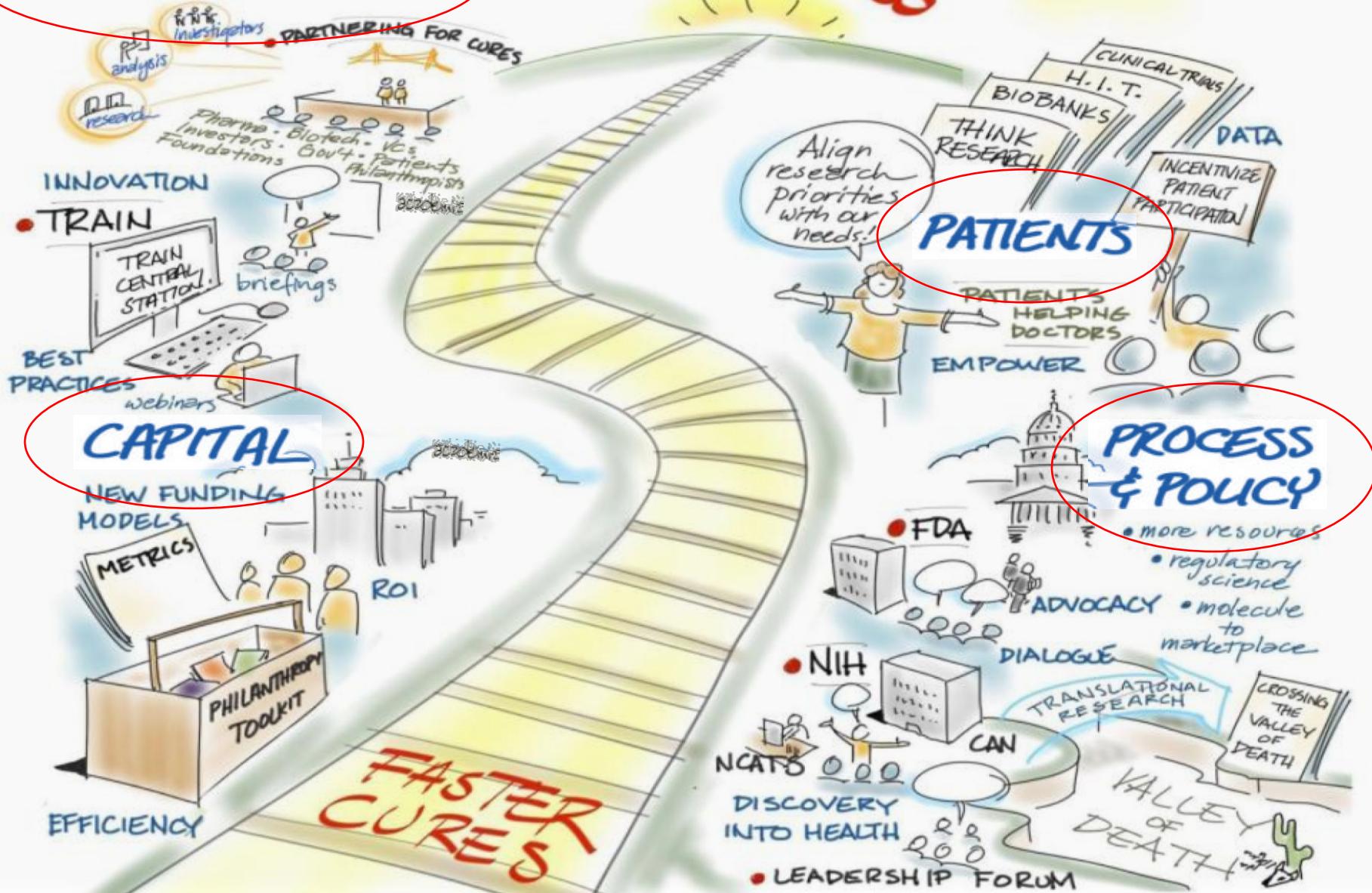
A look at the TRAIN clinical trial ecosystem.



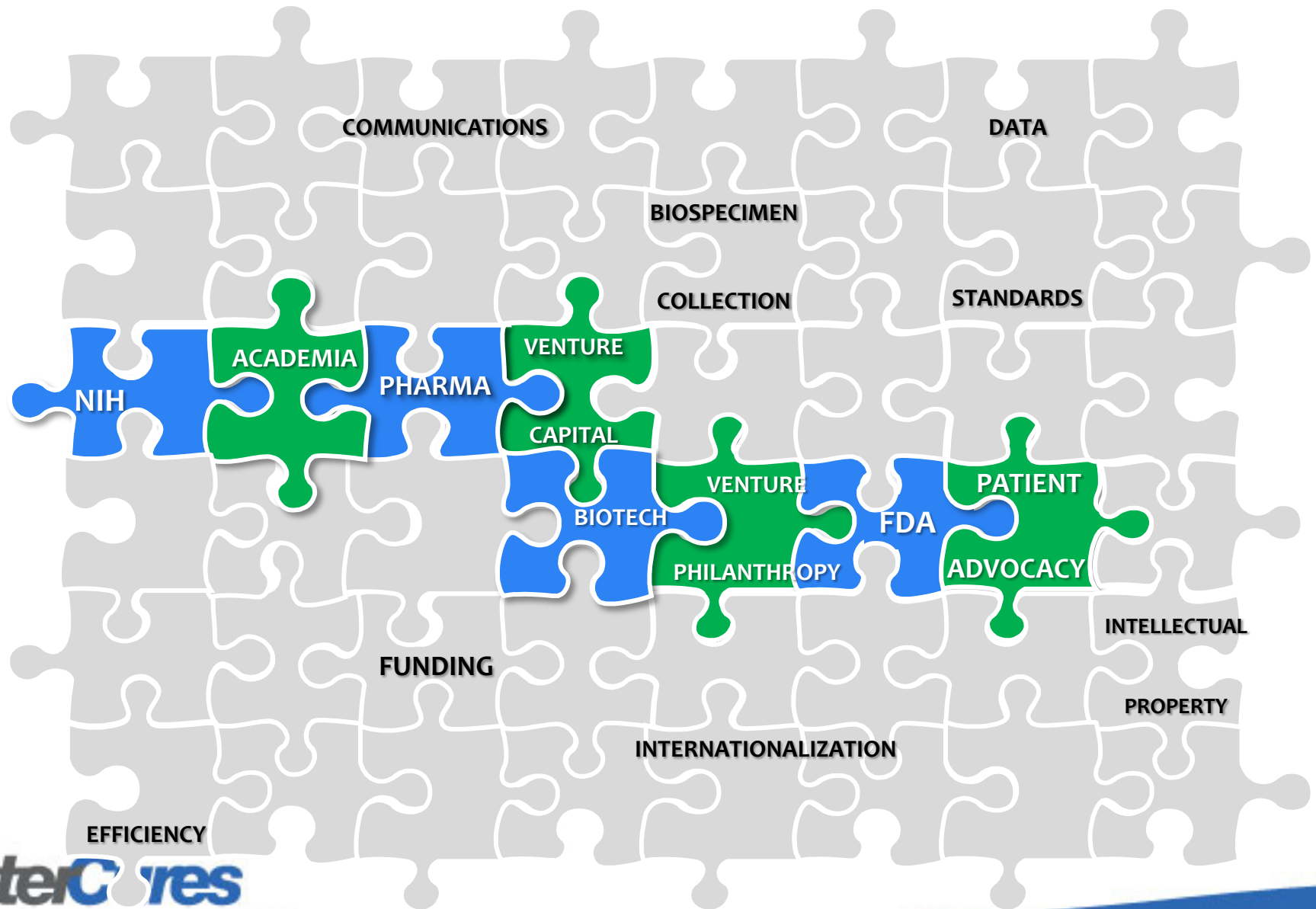
FasterCures

COLLABORATION

**MEDICAL
PROGRESS**



Challenges Require Collaboration



Finding Big Ideas in Small Spaces:

Venture Philanthropy's Innovative Approaches to Disease Research

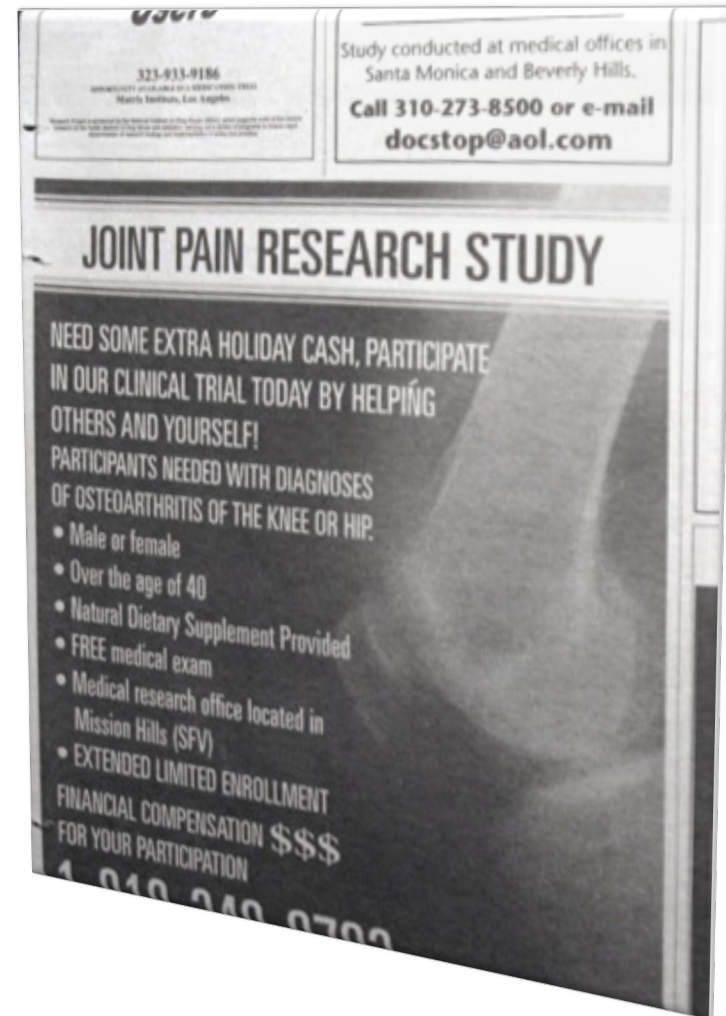


Venture Philanthropy in Medical Research



Patient Registry & Trial Databases

TRAIN organizations are changing the game of clinical trial recruitment.



Patient Registry & Trial Databases



- Launched officially in April 2012, has 14,000 volunteers and 200 trials in its database
- PD-specific tool to match patients to trials to ease patient participation and recruitment
- Overarching goal is to translate this tool to other diseases

Patient Registry & Trial Databases

Alpha-1 Foundation

Clinical Trial Recruitment: Core of Organizational Structure



The Alpha-1 Foundation structure stresses the importance of research in the process for all patients who join the organization.

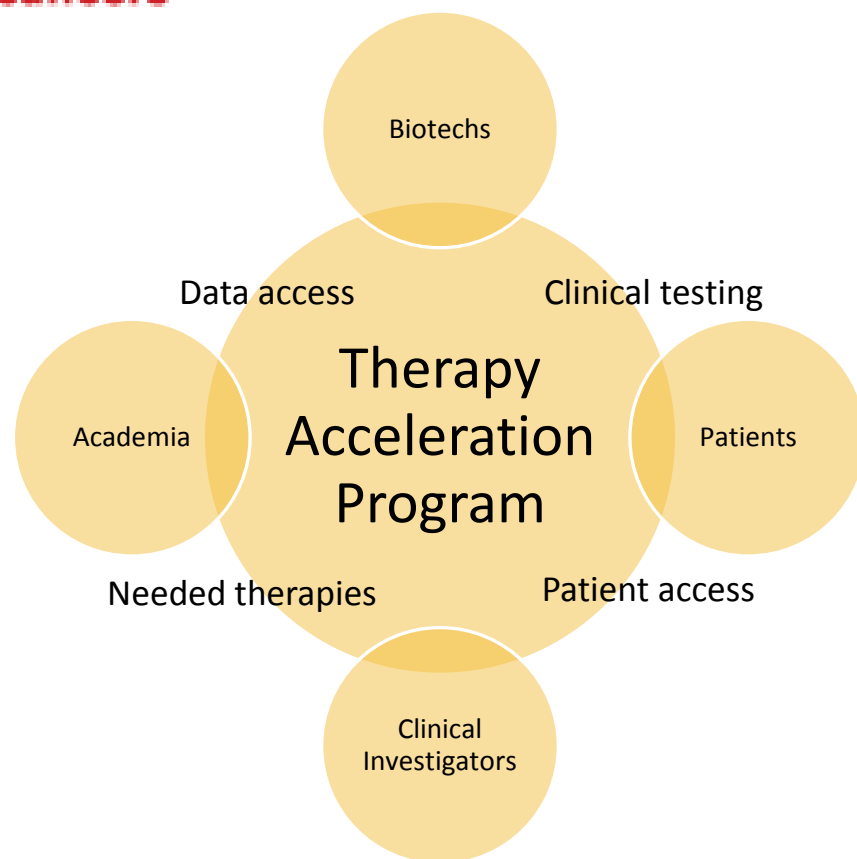
Other organizations with similar programs:

- Alpha-1
- ALSTDI
- Children's Tumor Fund
- Cystic Fibrosis Foundation
- Dr. Susan Love Research Foundation
- Foundation Fighting Blindness
- Leukemia and Lymphoma Society
- Juvenile Diabetes Research Foundation
- Multiple Myeloma Research Foundation
- Parent Project Muscular Dystrophy

Sponsoring Clinical Research



- Model venture philanthropy in clinical trial efforts:
 - Therapy Acceleration Program (TAP)
 - Targets therapies in areas of high unmet medical need
 - Funding to academic investigators and companies
 - Assists across all clinical phases

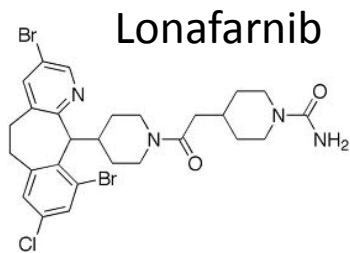




26 other organizations have similar programs...



Running a Complete Trial



- **Identified** the genetic mutation that causes progeria
- **Funded** preclinical research
- **Partnered** with Boston Children's Hospital
- **Completed** the trial for Lonafarnib in 2012
- **Continuing research support** through initiating a second trial

Clinical Centers

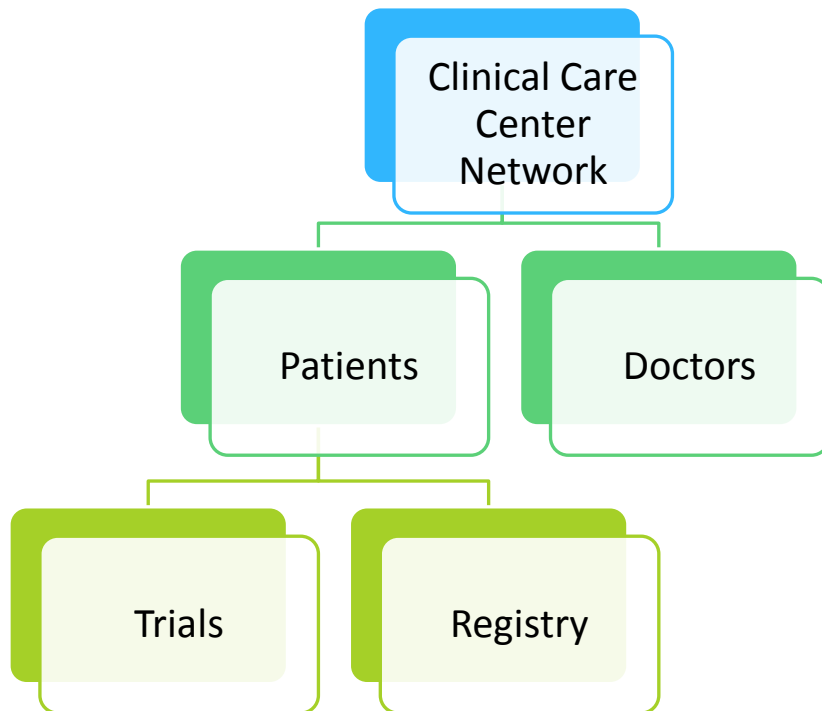
Trials run through a foundation's clinical center receives an implicit “seal of approval” from a respected and well known organization within the disease.



The Multiple Myeloma Research Consortium (MMRC):

- 16 member institutions
- Initiated 30 trials
- Begins trials 60% faster than industry
- Enrolls patients 10% faster than industry
- 8 drug studies in the final stage

Clinical Centers



RESULT: Kalydeco

Other organizations with similar programs:

- Alpha-1 Foundation
- ALS Therapy Development Institute
- Autism Speaks
- Translational Genomics Institute (TGen)

At the TGen clinical center, clinical trial participation is 60% while the national average is 3%.

These organizations are fixing the pipe.

Venture
philanthropies
are making
clinical trials
more efficient.

