TransCelerate BIOPHARMA INC.

ACCELERATING THE DEVELOPMENT OF NEW MEDICINES

Efficiency Enhancing Collaboration in Clinical Trials

Pharmaceutical R&D leaders identified collaboration as a key opportunity for generating industry-wide efficiencies

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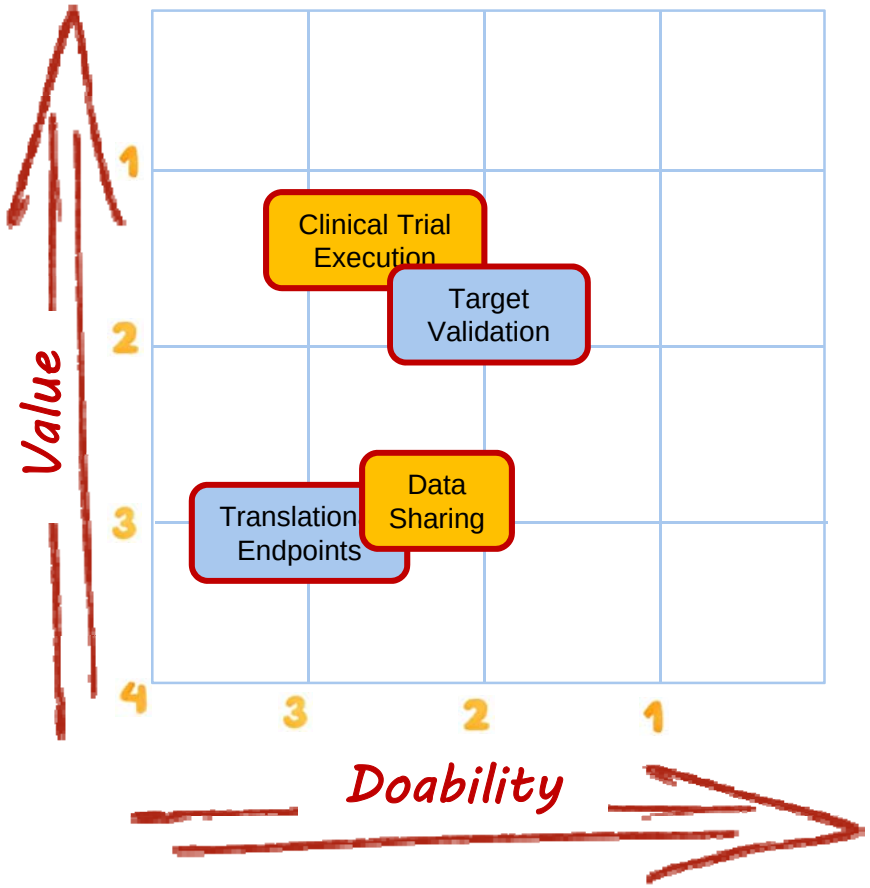
Conducted an industry survey on areas amenable to collaboration



The Charter Members of TransCelerate include Major Pharmaceutical Companies

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TBA (Board Member)

David Jordan (Operations Committee)
Divisional VP, Stats & Data Mgmt

Briggs Morrison (Board Member)
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Executive Director, Global Project Delivery

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Thor Voigt (Operations Committee)
Head of Global Clinical Ops, Biometrics & Data Management

Brian Daniels (Board Member)
SVP Global Development & Medical Affairs
Jonathan Zung (Chairman, Operations Committee)
VP, Global Development Operations

Patrick Vallance (Board Member)
President, Pharmaceuticals R&D
Lynn Marks (Corporate Secretary)
SVP, Clinical Platforms & Sciences
Pete Milligan (Operations Committee)
Director, Lead for SCD

Paul Stoffels (Interim Chairman of the Board)
Worldwide Chairman of J&J Pharmaceuticals
Martin Fitchet (Treasurer to the Board, Operations Committee)
Chief Operating Officer

Jan Lundberg (Board Member)
EVP of Science & Technology
Jeff Kasher (Operations Committee)
VP and COO Global Medical R&D

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Craig Lipset (Operations Committee)
Head of Clinical Innovation

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Carol Harris (Operations Committee)
Global Head Project & Functional Excellence

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President of Global R&D
Andy Lee (Operations Committee)
SVP, Head Global Clinical Operations

Nine new member companies joined TransCelerate in 2013

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Board Members

astellas

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SVP & Head of Global Development Operations
Nancy Sacco (Operations Committee)
Executive Director, Development Sciences/Strategic

biogen idec

Alfred Sandrock (Board Member)
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CUBIST PHARMACEUTICALS

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MerckSerono

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Kathleen Ford (Operations Committee)
Senior VP, Head of Global Clinical Operations

Forest Laboratories, Inc.

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Ulo Palm (Operations Committee)
SVP, Clinical Operations & Biometrics

Medgenics healing from within

Garry Neil (Board Member)
Global Head, R&D

Non-Board Members

ALLERGAN

Steve Johnson (Operations Committee)
SVP, R&D Business Services

SHIONOGI

Gareth Morgan (Operations Committee)
SVP, Portfolio Management, Global Development Office

ucb

Brigitte Koch (Operations Committee)
VP, Head Global Clinical Project Management

Not for profit entity created to drive collaboration as means to developing solutions for overcoming inefficiencies

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Our vision

To improve the health of people around the world by accelerating and simplifying the research and development of innovative new therapies.

Our mission

To collaborate across the global research and development community to identify, prioritize, design and implement solutions that drive the efficient, effective and high quality delivery of innovative new therapies.

Our core values

- Quality
- Transparency & Openness
- Trust & Integrity
- Collaboration
- Courage

A flat organization structure has been developed to manage projects and operational activities

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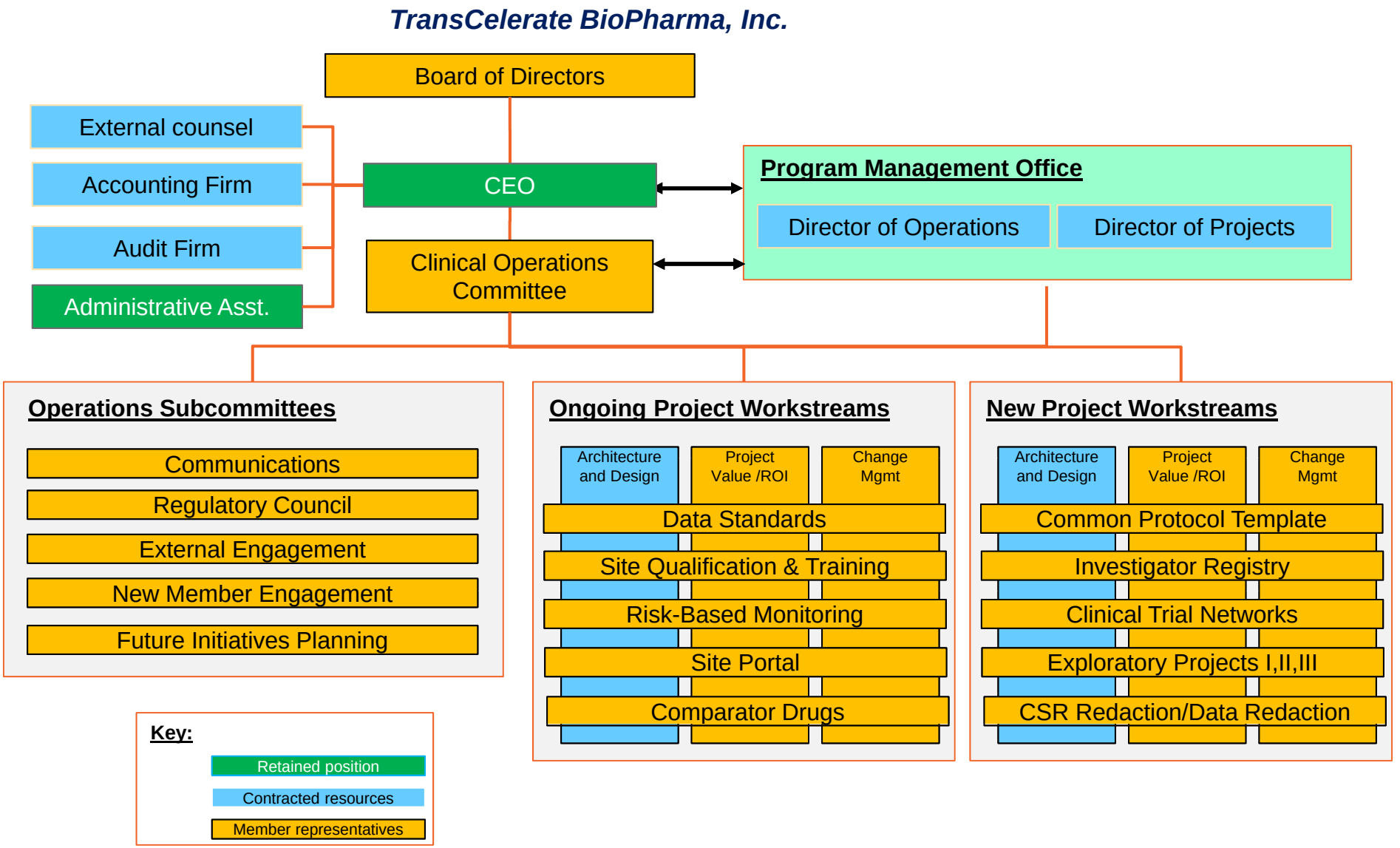
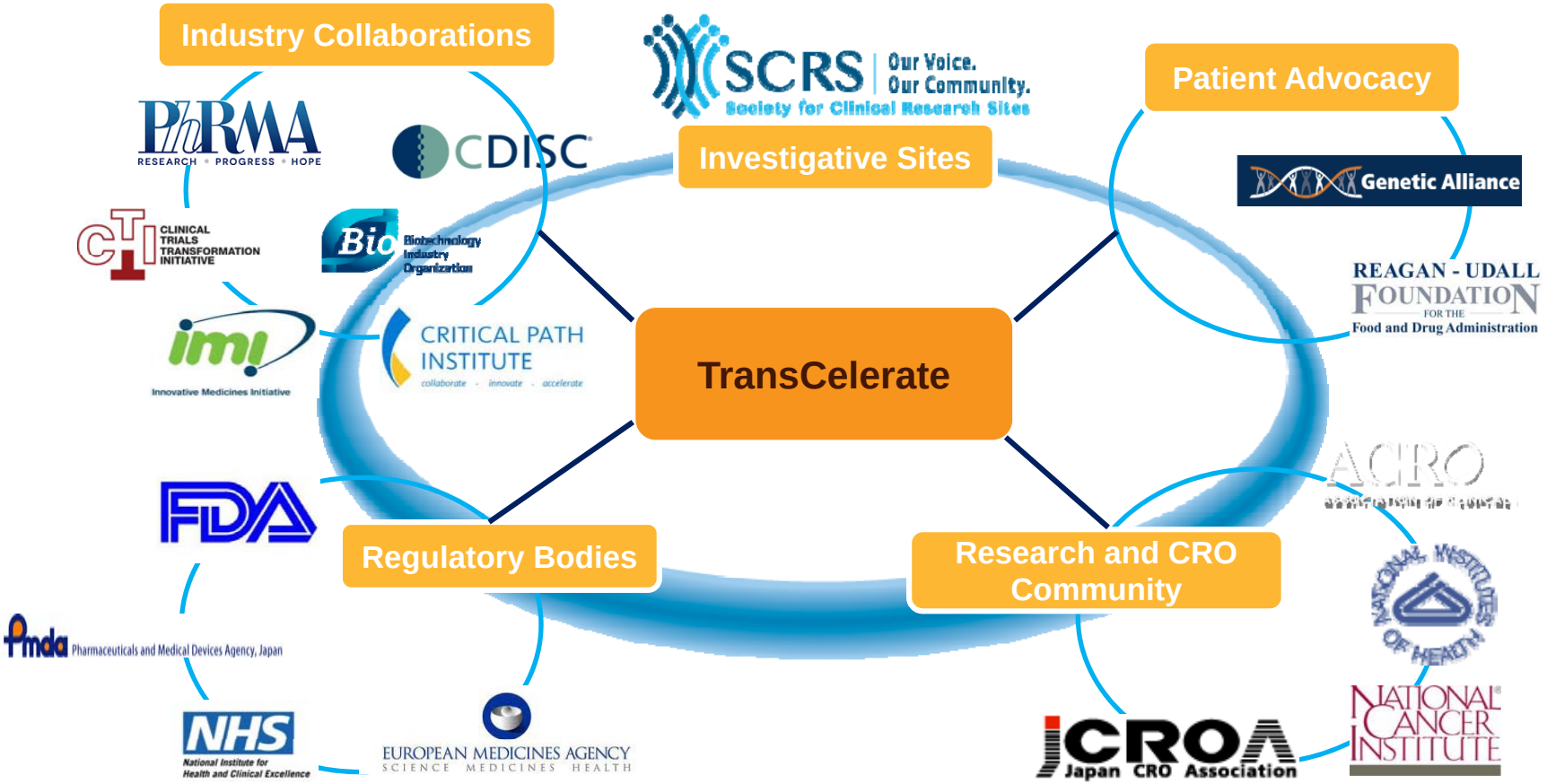


An entity that engages with the wider Clinical Ecosystem

The intent is not to recreate, but partner whenever feasible

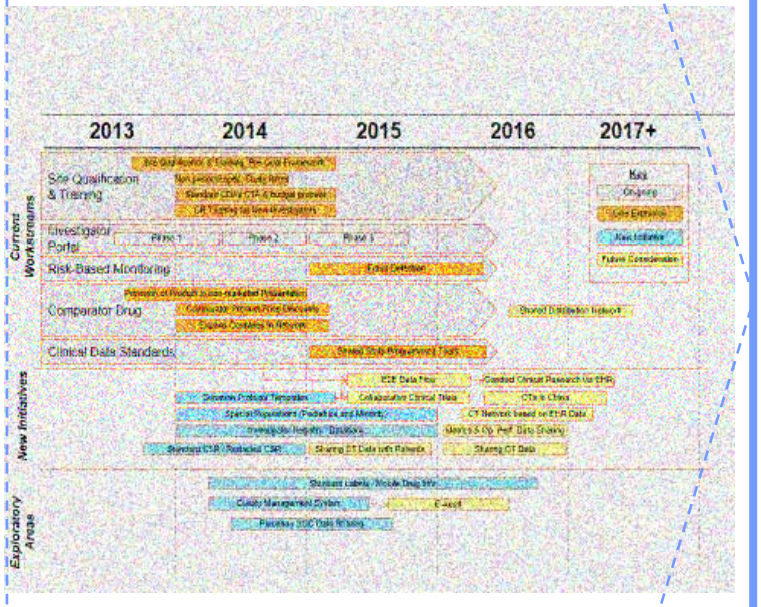


The future - a roadmap was created with the future state in mind

Current State

- Disconnected interfaces
- Manual processes and interventions
- Limited standardized solutions
- Lot of customization
- Rework
- Variable quality
- Wait time
- Missing information
- High costs
- Long cycle times
- Duplication of efforts

Roadmap



Future State

- Patient-centric clinical trial design
- End-to-end electronic data flow
- Seamless interfaces
- Automated
- Transparent
- Increased standardized solutions
- Less rework
- Quality by design
- Shorter cycle times
- Cost efficient
- Integration of Regulatory, Safety, and Medical Sciences
- Elimination of redundancies
- Conducting clinical trials together
- “Colossal Data Analytics”

Five opportunities were chosen for action based on industry readiness and ability to execute in 2013

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Prioritized Near Term Opportunities

1. **Standardized Approach for High-Quality, Risk-Based Monitoring**

Objective: Develop Guidelines for targeted, risk based clinical trial monitoring
Benefits: Improvement in data quality and patient safety for clinical trials; reduction in effort expended on low-value activities

2. **Shared Site Qualification and Training**

Objective: Program established for mutual recognition of GCP training and site qualification credentials
Benefits: Realization of improved quality of clinical sites and accelerated study start-up times

3. **Common Investigator Site Portal**

Objective: Establish a single, intuitive interface for investigators use across the industry
Benefits: Ease of use and harmonized delivery of content and services for investigators

4. **Clinical Data Standards – Efficacy** (*in partnership with CDISC)*

Objective: Accelerate current efforts underway through CDISC to establish efficacy data standards
Benefits: Increased quality of clinical data and enablement of industry end-to-end data flow

5. **Comparator Drugs for Clinical Trials**

Objective: Establish a supply network to source comparator drugs between companies for use in clinical trials
Benefits: Enhanced patient safety due to known product source and acceleration of study timelines

Projects have the shared goals of increased quality, patient safety & accelerated development timelines

Key Accomplishments to Date

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	Top Accomplishments in first year
1	Mobilized 10 companies to create TransCelerate, 10 new members joined in 2013
2	Created a lean and functional infrastructure of a not for profit entity
3	Initiated pilots, published SHARE environment and asthma standard released
4	Published the criteria for mutual recognition of GCP training
5	Published the framework and approach for risk based monitoring
6	Launched pilot studies for RBM across multiple member companies and TAs
7	Engaged multiple organizations – CTTI, SCRS, BIO, IOM, NIH, ACRO, IMI etc
8	First transaction of comparator drugs among member companies initiated

	Key Upcoming Milestones
1	Initiate clinical data standards development for additional therapeutic areas
2	Expand framework for streamlining site qualification beyond GCP training
3	Provide continued updates from learning's from RBM pilots to wider community
4	Launch the first release of the Shared Collaborative Technology Platform
5	Initiate new projects and expand scope on some existing projects

New Initiatives (1 of 2)

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Three New Initiatives Will Further Support the Goals of Increased Quality, Patient Safety and Accelerated Development Timelines			
Initiative	Unmet Need	Description	Benefit
Common Protocol Template	• Format of study protocols vary from company to company making interpretation difficult for study sites, IRBs, and regulators. • Study protocols have become increasingly complex as no agreed upon standards exists driving up cost and time. • Manual set-up of clinical systems based on non-standard “manual” protocols are time consuming, costly, and prone to error	Standardize the format of clinical protocols to ease interpretation & enable down-stream automation of many clinical processes. Develop industry-wide & regulator accepted standards for required protocol endpoints	• Higher productivity of sponsors, sites, IRBs, and regulators • Less costly and time consuming clinical trials • Enabler for downstream automated setup of clinical and operational systems & disclosure activities

New Initiatives (2 of 2)

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Three New Initiatives Will Further Support the Goals of Increased Quality, Patient Safety and Accelerated Development Timelines			
Initiative	Unmet Need	Description	Benefit
Investigator Registry	Sponsors invest significant time and money in identifying qualified investigators and setting up study sites	To create a shared repository of investigators to support targeted patient selection	Reduced cost and time of setting-up and running clinical trials
Special Populations Clinical Trial Networks (minority & pediatrics)	• Qualified investigators with adequate study patients are difficult to find for special populations – e.g. pediatric and minority • Studies in minorities and pediatrics are costly and lengthy • Efforts are put into repeatedly establishing a network for a single study only to disperse the network after study completion	Lead the development of global investigator networks for pediatric and minority populations including governance, investigator and patient registries, and technical infrastructure	• Faster development of new drugs in both pediatric and minority populations • Reduced costs of pediatric and minority trials

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Muchas gracias