President's Report

Year One Update on 5-Year Strategic Plan

Maria T. Millan, MD
President & CEO
June ICOC Board Meeting
June 29, 2023

CIRM
CALIFORNIA'S STEM CELL AGENCY

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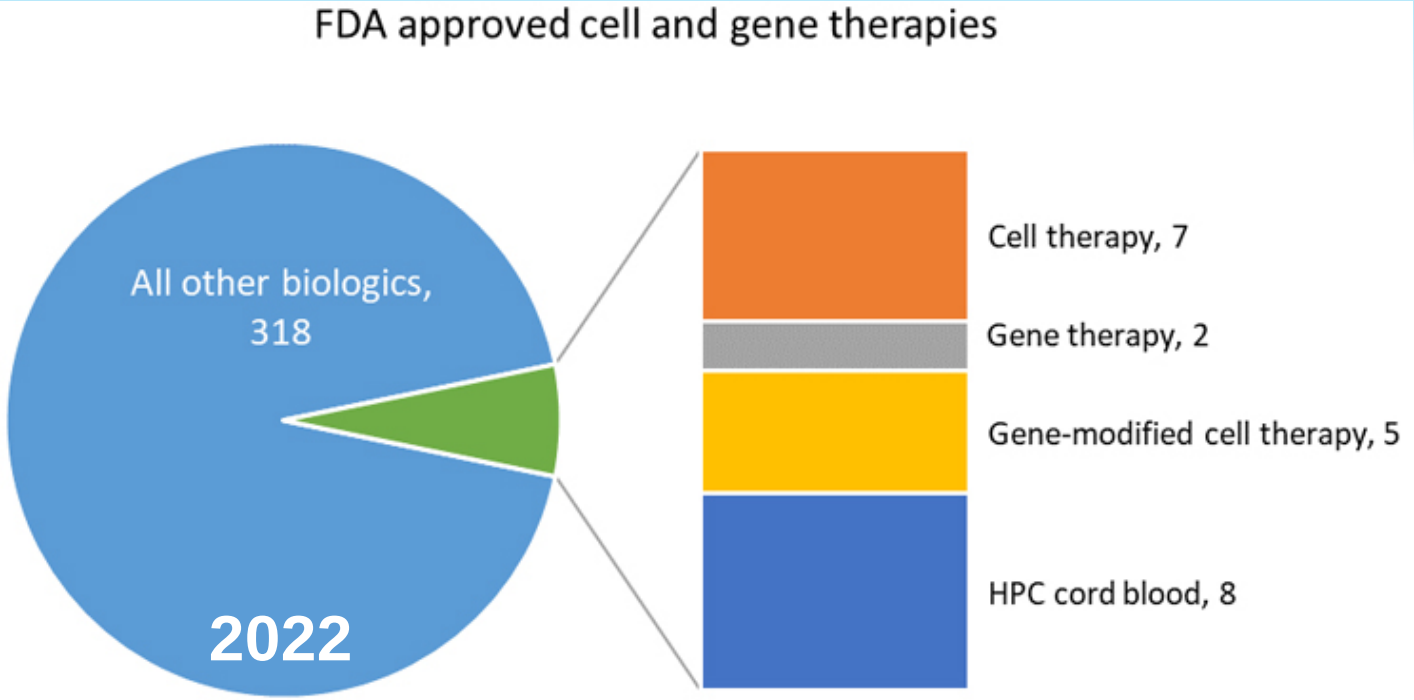
CALIFORNIA'S STEM CELL AGENCY

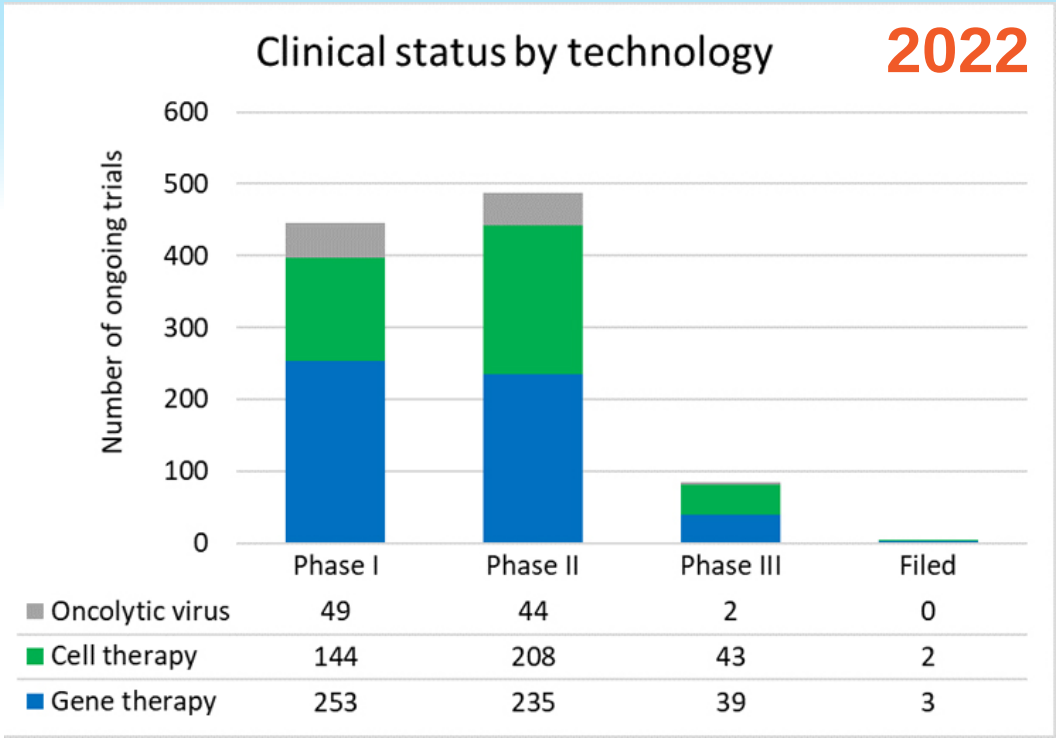
Real Life™

OUR MISSION

Accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world

Cell and Gene Therapy: State of the Field and CIRM





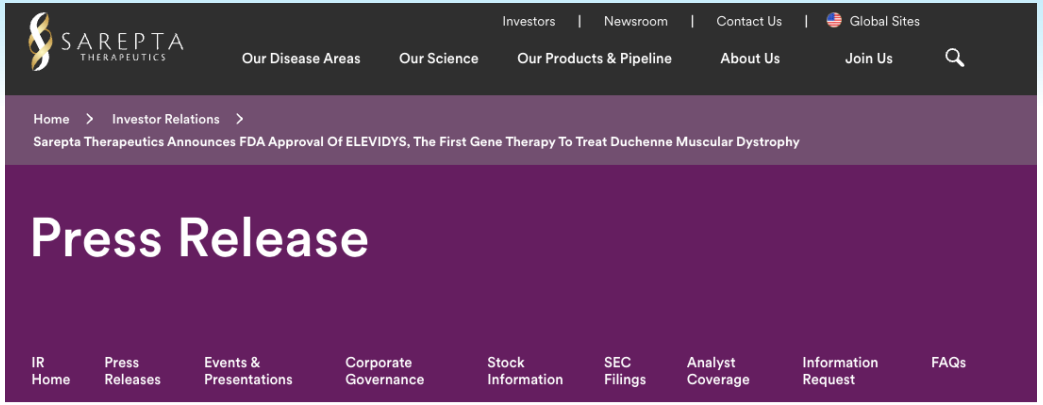
Source: FDA and Evaluate Pharma

To-Date

29 cell and gene therapies
5 expected in 2023
Sickle Cell Disease (2), Duchenne Muscular Dystrophy (2), Hemophilia A (1)

>90% CIRM **clinical** development programs in translational/early phase clinical trials

Recent Announcements point to Growing Potential for Cell and Gene Therapies



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PDF Version

Sarepta Therapeutics Announces FDA Approval of ELEVIDYS, the First Gene Therapy to Treat Duchenne Muscular Dystrophy

06/22/23 2:02 PM EDT

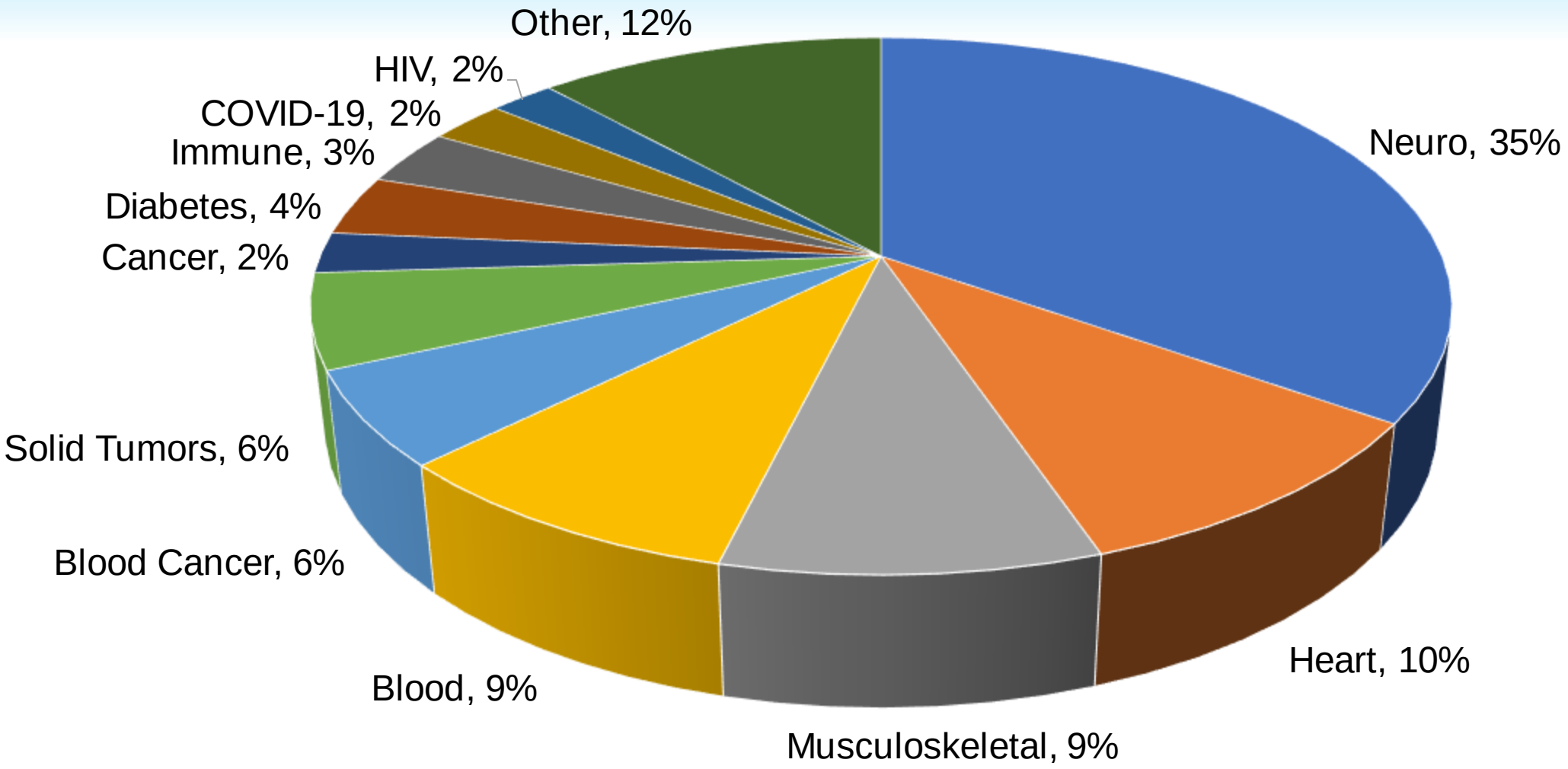
Neurona Therapeutics Presents One-Year Data on the First Patient Treated with NRTX-1001 Cell Therapy in an Ongoing Phase I/II Trial for Drug-resistant Focal Epilepsy

– Promising reduction (>95%) in seizure frequency at the key one-year post treatment endpoint in the first patient to receive NRTX-1001, memory improvements, and seizure-freedom since seven-months post-treatment

– Encouraging results seen in second patient dosed with NRTX-1001, where the cell therapy has been well-tolerated with continued reduction (>90%) in overall monthly seizure frequency at seven-months post-treatment

– Updated clinical and supporting preclinical data described in two oral presentations at the Annual Meeting of the International Society for Stem Cell Research (ISSCR)

CIRM's Diverse R&D Portfolio 2007-2023



Updated June 2023

Normalized to **total # of R&D awards**

CIRM Funding: ICOC-Approved Amounts / Number of Awards

Includes CLIN1 and CLIN2 (clinical trial) awards

Includes training and conference awards

	DISCOVERY	TRANSLATION	CLINICAL	EDUCATION	INFRASTRUCTURE
2004-Present	**$1.14B** **676**	**$573.2M** **133**	**$1.23B** **133**	**$431M** **300**	**$571M** **70**
Prop 14 Era	**$134M**** **91****	**$147M** **33**	**$296.4M** **37**	**$190.2M** **88**	**$125.3M** **19***
FY 22-23 **July 2022-June 2023**	**$58.7M** **33**	**$70.2M** **15**	**$164.5M** **22**	**$0.8M** **18**	**$9.9M** **5**

Updated June 2023

* # of awards does not include the supplementary Alpha Clinics awards

** Includes projects funded in prop 14 era but using prop 71 funds

FY 2022-2023 Progress on Prop 14 Strategic Plan

Addressing Barriers to Delivering CGT

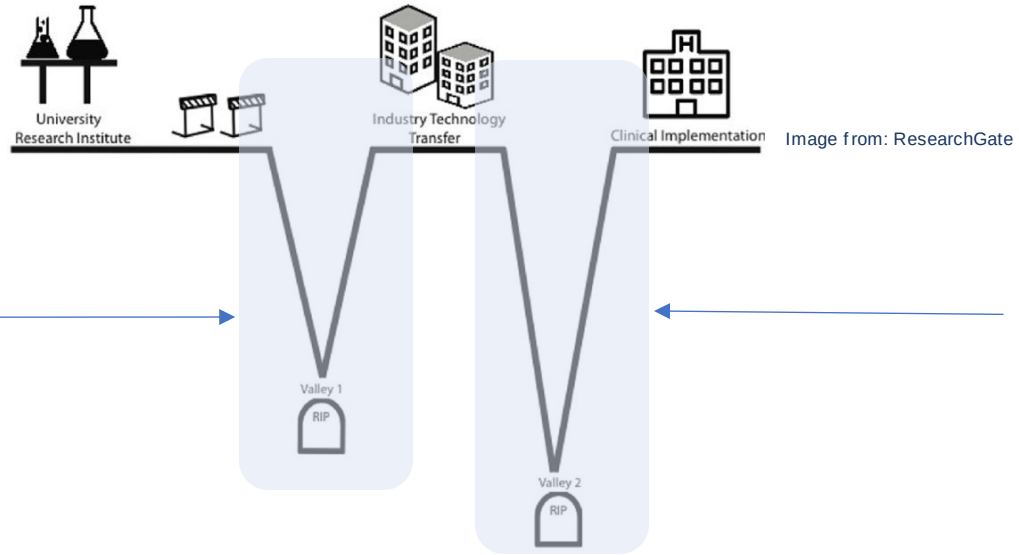


Image from: ResearchGate

De-risking projects

Supporting projects

- Opened Shared Resource Labs RFA
- Funded First Manufacturing Programs
- Launched Alpha Clinics
- Launched Education Projects

- Opened Patient Support Program RFA
- Community Care Centers Concept Development
- Initiated Roadmap for Access and Affordability

CIRM De-risks Programs in Challenging Financial Markets and Leverages Partnerships

May 22, 2023

Novartis Deal Boosts CIRM Approach: The Swiss Giant Plunks Down Nearly $88 Million for California-Financed Clinical Trial

CIRM funded the prior IND-enabling studies and ongoing clinical trial

This adds to the total $**24.4B** Industry investment in CIRM programs (10 deals between Jan 2022-June 2023)

Jordan with Dr. Cherqui (PI on Cystinosis Program)

A platform therapeutic modality developed through partnership


Don Kohn

Lentivirus gene therapy

- ADA-SCID
- XCGD
- Sickle cell disease
- Cystinosis

Initial Success in CGT is Also Our Problem Statement

ORIGINAL ARTICLE

Autologous Ex Vivo Lentiviral Gene Therapy for Adenosine Deaminase Deficiency

Donald B. Kohn, M.D., Claire Booth, M.B., B.S., Kit L. Shaw, Ph.D., Jinhua Xu-Bayford, D.I.P., Elizabeth Garabedian, R.N., Valentina Trevisan, M.D., Denise A. Carbonaro-Sarracino, Ph.D., Kajal Soni, B.Sc., Dayna Terrazas, R.N., Katie Snell, B.Sc., Alan Ikeda, M.D., Diego Leon-Rico, Ph.D., et al.

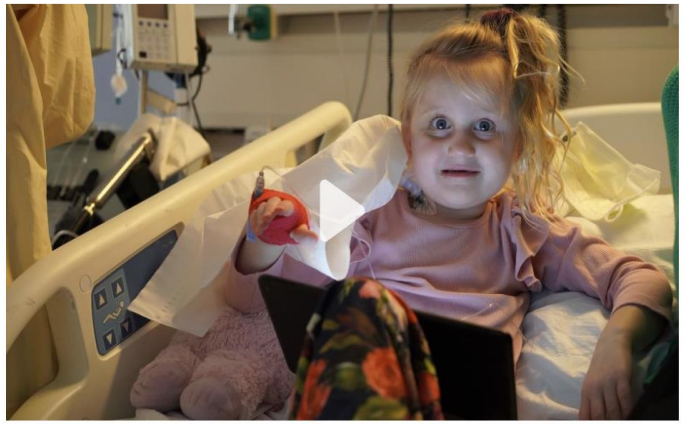
Treated as an infant
Evie at age 10
Cured of ADA-SCID

The NEW ENGLAND JOURNAL of MEDICINE

- 50 babies treated
- 100% event-free survival at 24 months
- 90% cured at 2 yrs and 100% at 3 yrs
- better outcomes than historical BMT registry groups

NEJM May 27, 2021

CNN health | Life, But Better | Fitness | Food | Sleep | Mindfulness | Relationships | Audio | Live TV | Log In



5-year-old with 'bubble boy disease' set for life-saving treatment

02:48 - Source: CNN

A drug company abandoned a treatment for 'bubble boy disease.' After a 5-year fight, this little girl is about to get it

By Elizabeth Cohen and Lauren Mascarenhas, CNN

Updated 5:01 PM EDT, Thu April 27, 2023

Solutions for Rare Diseases Present a Unique Opportunity for CGT Across All Indications

Opportunities:

Identify treatments for previously incurable and fatal diseases

Develop technology and platforms that can be applied to address broad indications

Develop delivery and access models that address the broad needs of CGT

Rare diseases affect

<200k Americans

NORD

Cumulatively, they affect

30 Million Americans

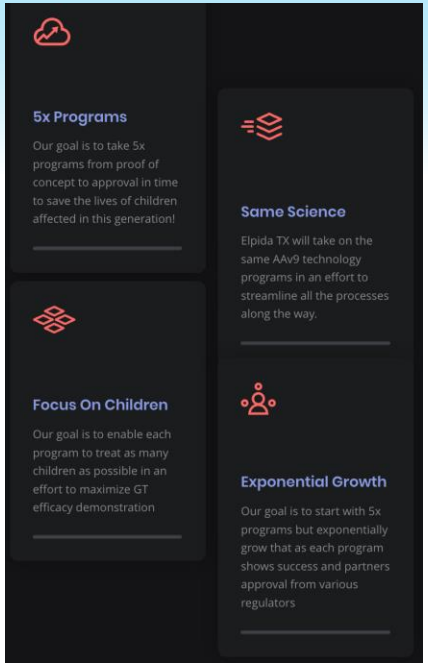
NORD

Globally, they affect

263-446 Million people

Wakap et al. 2020

Innovative Models to Deliver CGTs to Patients a Major Topic in 2023



Elpida Featured at LABEST

NY Times Dec. 9, 2022
Guest Essay (Fyodor Urnov)

N-of-1 Trials

Mila's Miracle Foundation presented at ASGCT

Consortia Models
ASGCT Panel
FNIH BeSpoke Gene Therapy Consortium for Pediatric Rare Disease

Pediatric CGT Access Think Tank

WASHINGTON

- Convened by Scientists looking for solutions on how to advance pediatric cancers & rare disease (CIRM, FDA & ARPA-H)
- Novel regulatory approaches
- Collaborations, Platform Trials, Point of Care Manufacturing
- Non-profit models, academic-based models, discussed

Partnership with Excellence

David Williams

ASGCT Founders Award

CIRM Awardee

(NHLBI Cure Sickle Cell Partnership)

Rayne Rouce

ASGCT Award for Excellence in Advancing DEI

CIRM GWG Member

Ysabel Duron

National Cancer Advisory Board by President Biden

CIRM Board Member

Patient Advocate

The Latino Cancer Institute

Joy Cavagnaro

ASGCT Catalyst Award

CIRM GWG Member

Helen Blau

Ernest McCulloch Lecturer

ISSCR Presidential Plenary

CIRM Awardee

Photo Credits: Courtesy of ASGCT 2023 & ISSCR 2023

Key Take-Aways

- CIRM's funding model has built a robust and diverse portfolio of discovery, therapeutics development, infrastructure and education programs.

- CIRM's 5-year Strategic Plan (2022-2027) was launched with the goal of advancing transformative science to clinical trials and therapies and to make them accessible to all in need.

- The CIRM funding model and strategy are adaptable to real-time advancements in field, emerging priorities and opportunities to advance CIRM's mission

  (e.g. in rare disease, CNS disease, untreatable solid cancer and other priority areas under consideration)

How We Currently Build our Portfolio and Achieve our Strategic Objectives Through Funding

Dr. Gil Sambrano