



Takeda – Pittsburgh Alliance

Request for Proposals Information Packet



Fall 2020

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Better Health, Brighter Future

Request for Proposals: Contacts

Questions?

- Contact Amy Brunetto Phillips for the short proposal template and general questions
 - azb5@pitt.edu
- IP questions? Contact your licensing manager

Who we are

PUTTING PATIENTS FIRST FOR OVER TWO CENTURIES

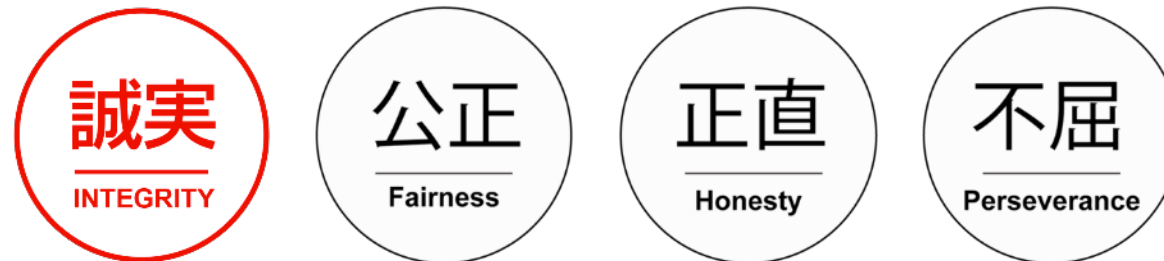
Takeda is a global, values-based, R&D-driven biopharmaceutical leader headquartered in Japan, committed to bringing **Better Health and a Brighter Future** to patients by translating science into highly-innovative medicines.



Better Health, Brighter Future

Values

Established by our founding spirit and integral to every part of our business, **Takeda-ism** and **our priorities** guide us in our efforts to achieve Vision 2025.



TAKEDA-ISM

OUR PRIORITIES

We make decisions and take action by focusing on our four priorities, in this order:

- 1 Put the patient at the center** >
- 2 Build trust with society** >
- 3 Reinforce our reputation** >
- 4 Develop the business**

Patient-Driven and Science-First in 3 Core Areas

INNOVATIVE BIOPHARMA



ONCOLOGY



RARE DISEASES



NEUROSCIENCE



GASTROENTEROLOGY

PLASMA DERIVED THERAPIES



Complementing our
rare disease focus

VACCINES BUSINESS UNIT



Differentiated
Dengue vaccine

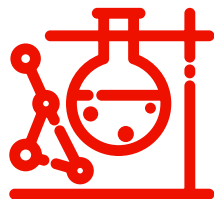
We Have a Differentiated R&D Engine

A Diverse and Dynamic Pipeline¹



200+

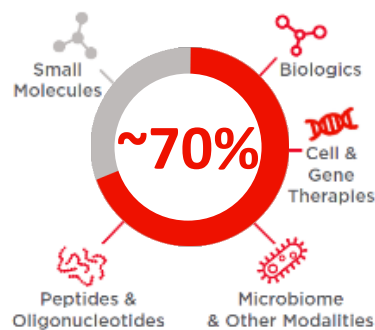
ACTIVE PARTNERSHIPS



~40

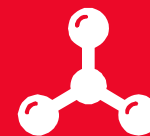
NEW MOLECULAR
ENTITY CLINICAL
STAGE ASSETS

DIVERSIFIED
MODALITIES
IN RESEARCH



PIPELINE WITH
ORPHAN DRUG
DESIGNATION¹

Expanding Critical Capabilities



CELL THERAPY



GENE THERAPY



DATA SCIENCES

¹ Pipeline as of November 14, 2019

¹ 31 Orphan Drug Designations in at least one indication for assets in Phase 1 through LCM in 2019 versus 15 in 2018

Our Approach to Research Collaboration



- World-class, cutting edge discovery biology
- Deep investigator community
- Broad expertise across therapeutic areas
- Patient focused, patient experience
- Entrepreneurial spirit



- Patient-centric approach
- Deep therapeutic areas expertise
- World-class translational capability
- Clinical development & operational expertise
- Commercialization strategy

Joint effort Collaboration

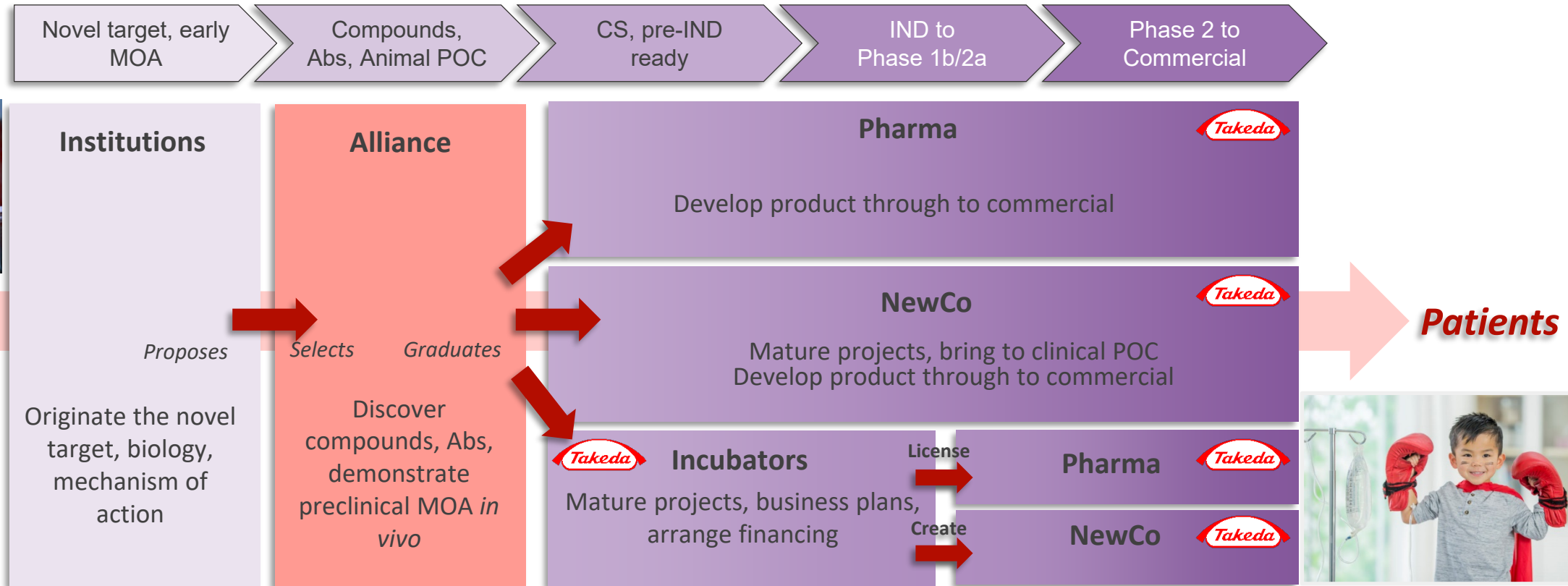


Design and translate early research to therapeutics

Alliances Integrate into a Discovery Pipeline to Develop Drug Candidates



Innovation



Benefits to Investigators

- A committed therapeutic development partner
 - Opportunities to License projects to Takeda or form new companies
- The opportunity to transform an idea to a product
- Commitment to joint publications and generation of new research tools
- A share in the future success of the project

Application Process

- Short proposal stage
 - 3 page, non-confidential, template provided
 - Reviewed and selected by Joint Steering Committee to submit a full proposal
- Full proposal stage
 - 5-6 page, confidential, template provided
 - Investigator to be paired with Takeda scientists to co-develop full proposal
 - Full proposals include goals, research plans, timelines, expected financial and in-kind resources from each Party, and criteria for go-no go milestones
- Approved Project stage
 - Project team assembled
 - Project plan and decision points established

Criteria for Project Selection

- Transformative approach, platform, or biology
- Clearly defined proposal with a testable hypothesis
 - Specify activities to be performed in investigator lab vs those by Takeda team
- Insight and/or expertise in the disease biology
- Availability of *in vitro* and *in vivo* models to robustly test the therapeutic hypothesis

Request for Proposals Timeline (draft)

Event	Dates
Takeda Day kick off event	Tuesday, September 15, 2020
Takeda Presentation to Pitt faculty	Thursday September 24th
Short proposal application due to OEP via Formstack	Thursday, October 15, 2020
Notification of selected investigators to co-develop proposal w/ Takeda teams	Monday, January 4, 2021
Full proposal application due date	Monday, March 15, 2021
Oral presentations; review and selection by JSC	Mid April, 2021
Award notification (subject to refinements of project plan & budget)	Monday May 3 2021
Project launch	End May, 2021

Summary of Takeda Areas of Interest



EARLY INNOVATION & PLATFORMS	RARE DISEASES	ONCOLOGY	GASTROENTEROLOGY	NEUROSCIENCE
Gene and Cell Therapy Technologies	Lysosomal Storage Disorders	I/O Cell Engagers & Cell Therapy	IBD inflammation	Nervous system protection/repair
Antibodies, Biologics, Small Molecules	Rare Hematology	I/O Cold to Hot	Motility disorders	Precision medicine approach
Small Molecule-RNA binding	Inborn Metabolic Disorders	Tumor microenvironment Immune cells: B, NK, and T eff cells, macrophages	Liver disorders (especially F3, F4 NASH)	BBB shuttle/delivery
Delivery technologies (LNPs, Exosomes, etc.)	Musculoskeletal, Neuromuscular	Target ID (MM, MSS-CRC, TNBC, Pancreatic, AML, lymphoma)	Microbiome	Synergistic Pharmacology (Polypharmacology)
Protein Degradation	Rare Immunology	Patient sample access	Genetic linked diseases	Genetic linked diseases
Translational Medicine	Complement-mediated	Delivery, Armoring Payloads	Cell Therapy	Any other emerging therapeutic approaches
AI applied to patient and clinical data, Data Science, RWE	Platforms: delivery, cell/gene therapy, immune tolerance	Translational models	Drug Delivery	

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Rare Disease Areas of Interest



Drug Candidates for the Treatment of Any Orphan Disease or for Orphan Subpopulations of Larger Diseases

- Lysosomal Storage Diseases
- Inborn Metabolic Disorders
- Musculoskeletal - Neuromuscular
- Rare Hematology
- Rare Immunology
- Complement-mediated
- Technologies/Platforms promoting novel and innovative approaches for:
 - Drug delivery
 - Cell and Gene therapy
 - Targeting disease-specific/metabolic-specific pathway(s)
 - Targeting specific tissues/organs i.e. BBB or muscle
 - Immune tolerance, novel immune modulator MOAs for Ab-mediated autoimmune diseases

Gene Therapy Areas of Interest



Expand Access to new Gene Therapy Platforms Through Collaborations

- Enhance viral AAV platform (improve muscle, CNS/including cell-specific targeting)
 - Capsid libraries
 - Regulatory, tissue specific, & ubiquitous promoters
- Build non-viral gene delivery capabilities
 - Targeted delivery of nucleic acid, gene editors, transgene enhancers, promoters peptides/proteins
 - LNP, exosomes, cell-penetrating peptides
 - Tissue and specific delivery
- Computational biology
- Immunological assays
- Protein engineering
- Gene editing
- Build ex-vivo/cell therapy, T-reg cells

Oncology Areas of Interest

Immuno-Oncology



'Cold-to-Hot' Environment

- Target immune and non-immune mediated immunosuppressors
- Enhanced Cytokines
- Nextgen approaches to *in situ* engineering of immunocytes
- OV technologies complementary to pipeline



Redirected Immunity

- Augment cell therapy pipeline
- Nextgen approaches to *in situ* engineering of T, NK, $\gamma\delta$ T cells
- Novel engager platforms beyond T cells

Core Malignancies



Hematologic and Lung Cancers

- Novel targets/ approaches
- Nextgen lung discovery approaches
- Clinical assets in core areas

Emerging Biology



Next Wave Efforts

- Novel modalities to unlock intractable targets
- Innovative targets not restricted to immuno-oncology

Foundational Capabilities



Platform Build

- Expand translational science toolbox
- Broaden biologic scaffold repertoire
- Enable efficient and specific tumor localization

Gastroenterology Areas of Interest



Gut Inflammation

- IBD (Crohn's, UC), Celiac
- Barrier integrity and mucosal healing
- Microbiome therapeutics (small/large molecules, consortia)



Liver Disease

- Advanced/late stage liver disease (F3, F4 NASH only), anti-fibrosis drivers,
- Hepatocyte rejuvenation, regeneration, replacement
- Primary sclerosing cholangitis
- Rare disease and genetic linked: Wilson's



Other areas of interest

- Cell and gene therapy
- Access to patient samples and/or patient cohorts
- Drug delivery with specificity and selectivity



Motility Disorders

- Gastroparesis, nausea and vomiting indications
- Enteric nervous system: Hirschsprung's disease and esophageal achalasia (opportunity for cell therapy), fecal incontinence



Translational & Clinical

- Clinically meaningful targets, biomarkers, models, assays, endpoints
- Causal validation of targets, therapeutic modulation that recapitulates human biology
- Patient progression and disease outcome predictions
- Patient identification and stratification strategies



Areas of no interest

- Metabolic NASH/NAFLD, Hepatitis, IBS, visceral pain, Parkinson's- and MS-constipation, chronic idiopathic constipation, pancreatitis, phage therapeutics, single strain bacterial therapeutics, engineered bacteria

Neuroscience Areas of Interest



Nervous System Protection/Repair

- Healthy aging (e.g. sleep/circadian rhythm, waste clearance, BBB integrity, anti-chronic inflammation, organelle homeostasis, DNA damage, sensory system)
- Address functional disturbances of brain associated with medication or acute/chronic insult (e.g. chemobrain, POCD, CTE)
- Neurological aspects of neuromuscular disorders



BBB Shuttle/Delivery

- Efficient delivery of therapeutics across blood brain barrier
- Platform technologies enabling cell type specific intervention in the brain



Precision Medicine

- Therapeutic approach for a stratified sporadic disease population
- Stratification/therapeutic approach cutting across current disease classification
- Wearable/non-invasive devices and sensors to collect digital biomarkers toward patient stratification and/or monitoring
- Enablement of early intervention/preemptive therapy through identifying right patient population



Synergistic Pharmacology (Polypharmacology)

- Validated target and pathway for diseases in Neuroscience elucidated from artificial intelligence approach
- Therapeutics to be combined with therapeutic devices (e.g. TMS/TES) to improve the outcome



Genetic-Linked Diseases

- In Neuroscience field