MARCH 15, 2023

CP-RND: AN INTRODUCTION TO THE PATIENT COMMUNITY

An opportunity to share an overview of CP-RND and gain insight from the patient community.

CP-RND
CRITICAL PATH FOR RARE NEURODEGENERATIVE DISEASES
CRITICAL PATH INSTITUTE

c-path.org/cp-rnd

WELCOME & HOUSEKEEPING

Welcome to the Critical Path for Rare Neurodegenerative Diseases patient engagement webinar. A few housekeeping items before we get started:

Q&A

Place all questions in the Q&A Chat box.

Send questions to “All Panelists” to ensure they are seen.

LINES ARE MUTED

All participant lines are muted to reduce background noise.

RECORDING

This presentation is being recorded and will be made available on the C-Path YouTube channel.

CONTACT INFO

CP-RND@c-path.org for more information or questions following the webinar.

AGENDA

This webinar is an opportunity to share an overview of CP-RND and gain insight from the patient community

Time	Topic	Speaker(s)
9:30 - 9:40 am	Welcome/Opening Remarks	Jacqueline Corrigan-Curay (FDA) Klaus Romero (C-Path)
9:40 – 10:00 am	CP-RND Overview	Collin Hovinga (C-Path)
10:00 – 11:00 am	Panel Discussion/Q&A	Patient Advocacy/FDA/C-Path/NIH
11:00 – 11:15 am	Closing Remarks	Collin Hovinga (C-Path)

Critical Path Institute is supported by the Food and Drug Administration (FDA) of the Department of Health and Human Services (HHS) and is 55% funded by the FDA/HHS, totaling $17,612,250, and 45% funded by non-government source(s), totaling $14,203,111. The contents are those of the author(s) and do not necessarily represent the

JACQUELINE CORRIGAN-CURAY, JD, MD

Principal Deputy Center Director,
CDER, FDA

KLAUS ROMERO, MD, MS, FCCP

Chief Science Officer; Executive
Director of Clinical Pharmacology,
Critical Path Institute

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COLLIN HOVINGA, PHARMD, MS, FCCP

Vice President, Rare and Orphan Disease Program, C-Path

Dr. Hovinga oversees C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform and its Critical Path for Rare Neurodegenerative Diseases public-private partnership.

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CP-RND OVERVIEW

- Introduction to Critical Path Institute (C-Path)
- Overview of Critical Path for Rare Neurodegenerative Diseases (CP-RND)
- Discuss Next steps and mechanisms for involvement

OVERVIEW OF C-PATH

Mission

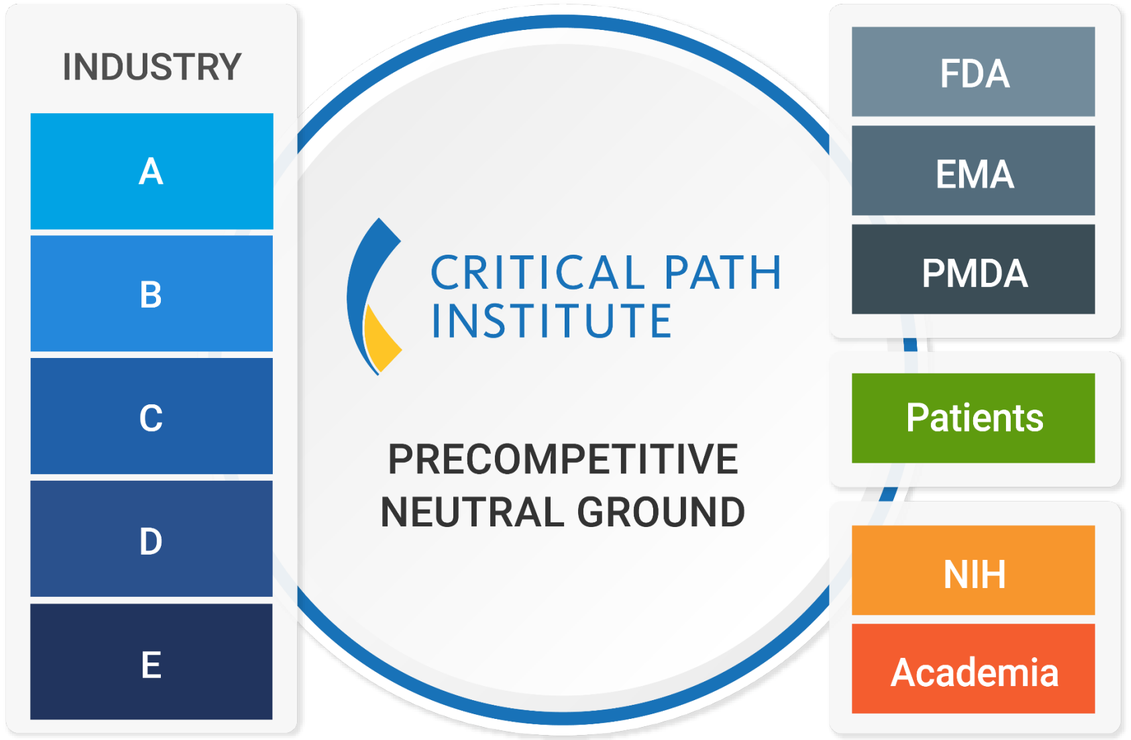
Accelerating the path to a healthier world. C-Path builds solutions that remove bottlenecks in the drug development process.

Vision

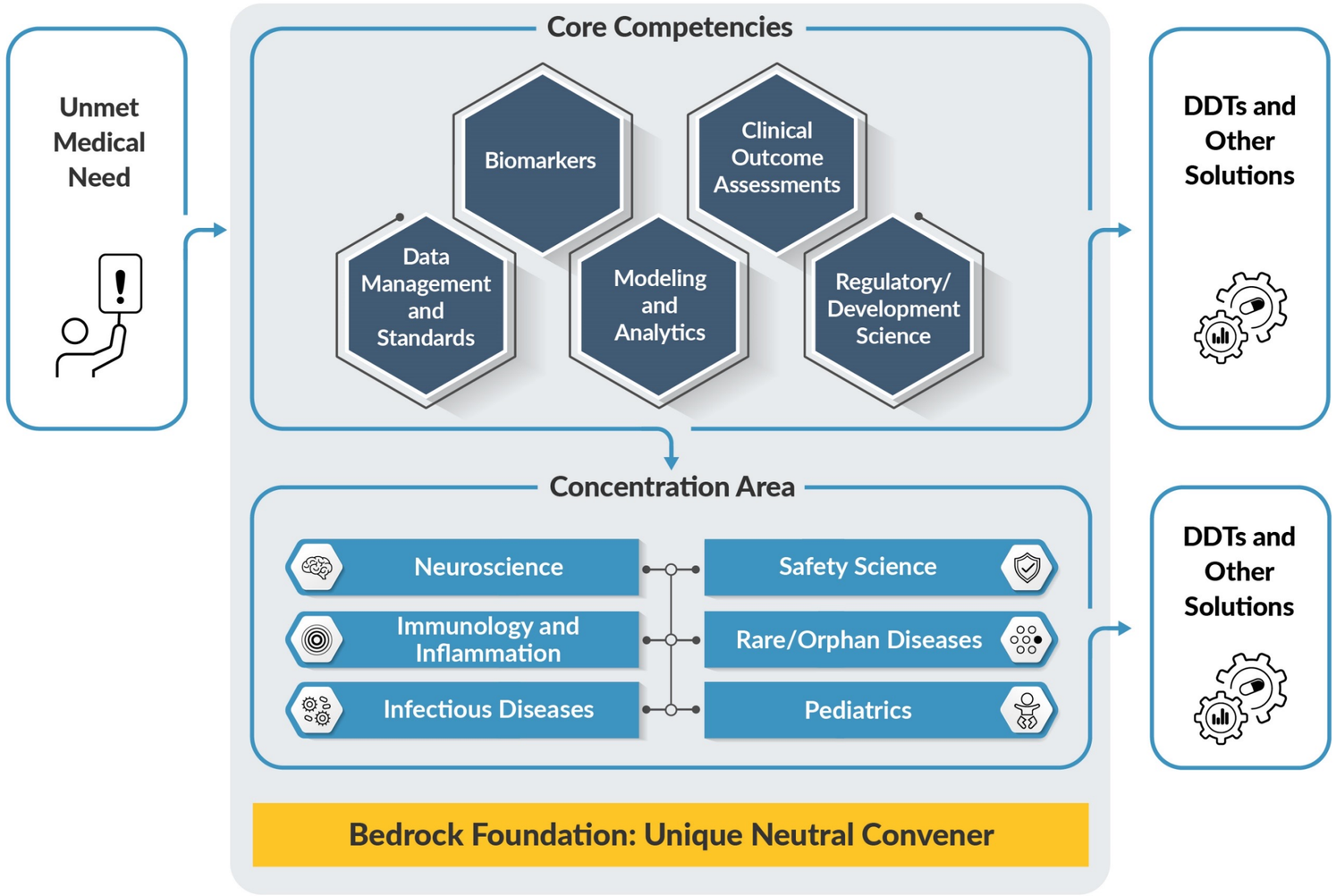
Critical Path Institute removes competitive blocks in the medical product development process. **This makes C-Path an indispensable partner for researchers, patients and regulators.** Our role as a neutral convener in the medical and scientific industry accelerates therapies for patients in need.

C-PATH, A PRECOMPETITIVE NEUTRAL CONVENER

- Act as a trusted, neutral third party
- Public-Private Partnerships
- Convene scientific consortia of patients, industry, academia, and government for sharing of data and expertise
  - ✓ The best science
  - ✓ The broadest experience
  - ✓ Active consensus building
  - ✓ Shared risk and costs
- Enable iterative EMA/FDA/PMDA participation in developing new methods to aid in the assessment the safety and efficacy of medical products
- Official regulatory endorsement of novel methodologies and drug development tools (DDTs)



CORE COMPETENCIES & CONCENTRATION AREAS



ACTIVE C-PATH CONSORTIA/PROGRAMS

Active Consortia/Programs

BmDR	Biomarker Data Repository
CDRC	Cure Drug Repurposing Collaboratory
CPAD	Critical Path for Alzheimer's Disease
CPP	Critical Path for Parkinson's Disease
CPTA	Critical Path to Therapeutics for the Ataxias
CPTR	Critical Path to TB Drug Regimens
CP-SCD	Critical Path for Sickle Cell Disease
DCC	Data Collaboration Center
D-RSC	Duchenne Regulatory Science Consortium
eCOA Consortium	Electronic Clinical Outcome Assessment Consortium
ERA4TB	European Regimen Accelerator for Tuberculosis*
HD-RSC	Huntington's Disease Regulatory Science Consortium
INC	International Neonatal Consortium
MSOAC	Multiple Sclerosis Outcome Assessment Consortium
PKDOC	Polycystic Kidney Disease Outcomes Consortium
PredicTox KE	PredicTox Knowledge Environment
PRO Consortium	Patient-Reported Outcome Consortium
PSTC	Predictive Safety Testing Consortium
QuantMed	Quantitative Medicine
RDCA-DAP	Rare Disease Cures Accelerator- Data and Analytics Platform
RD-COAC	Rare Disease Clinical Outcome Assessment Consortium
T1D	Type 1 Diabetes Consortium
TOMI-T1D	Trial Outcome Markers Initiative in T1D Consortium
TTC	Transplant Therapeutics Consortium
TRxA	Translational Therapeutics Accelerator
UNITE4TB	Worldwide Accelerator for Tuberculosis*
CP-RND	Critical Path for rare neurodegenerative diseases
CPA-1	Critical Path for Alpha-1 antitrypsin deficiency (pre-consortium)
CPLD	Critical Path for Lysosomal Disorders (pre-consortium)

* C-Path Ltd.

EXAMPLES, A SUCCESS STORY - REGULATORY FIRSTS

C-PATH REGULATORY SUCCESSES

ALZHEIMER'S DISEASE

- FDA & EMA endorsed AD clinical trial simulation tool
- EMA qualified model-based AD biomarker
- FDA & EMA letters of support
  - Model-based AD biomarkers and pre-dementia clinical trial simulator

MULTIPLE SCLEROSIS

- EMA qualified PerfO measure
  - Test battery for all forms of MS

POLYCYSTIC KIDNEY DISEASE

- EMA & FDA model-based qualified Total Kidney Volume (TKV) imaging biomarker
- FDA letter of support
  - TKV imaging biomarker
- FDA designated reasonably likely surrogate marker for PKD trials (TKV)

PREDICTIVE SAFETY TESTING

- EMA, FDA & PMDA qualified non-clinical kidney safety biomarkers
- FDA qualified clinical kidney safety markers
- Six FDA & EMA letters of support

PARKINSON'S DISEASE

- FDA letter of support
  - PD imaging biomarker
- EMA qualified model-based PD imaging biomarker

TUBERCULOSIS

- EMA qualified translational drug development platform

PATIENT-REPORTED OUTCOME MEASURES

- FDA COA qualification
- Symptoms of Major Depressive Disorder Scale
- Non-Small Cell Lung Cancer Symptom Assessment Questionnaire
- Asthma daytime and nighttime symptom diaries

TYPE 1 DIABETES

- EMA letter of support for model-based islet autoantibodies biomarker for trial enrichment

FDA

- 6 Qualification Decisions
- 1 Fit-for-Purpose Endorsement
- 7 Letters of Support

EMA

- 7 Qualification Decisions
- 7 Letters of Support

PMDA

- 1 Qualification Decision

CP-RND PUBLIC PRIVATE PARTNERSHIP (PPP)

- Accelerating Access to Critical Therapies for ALS Act (ACT for ALS): FDA awarded C-Path a grant to establish a PPP involving FDA/NIH, patients/advocates, researchers and industry aimed at advancing research in ALS and other rare neurodegenerative diseases.

  - ✓ Ataxias- CPTA
  - ✓ Huntington's Disease-HD-RSC
  - ✓ Amyotrophic lateral sclerosis (ALS)
  - ✓ Others

- A critical role in this model is the consistent input and engagement with patients, caregivers and the advocacy community.
- Collaboration with researchers and industry experience will help define scientific evidence and gaps in drug development.

CP-RND ORGANIZATION

CP-RND PPP Government-Partners

CP-RND
CRITICAL PATH FOR RARE NEURODEGENERATIVE DISEASES
CRITICAL PATH INSTITUTE

CP-RND Stakeholders

FDA POC

NIH/FNIH POC

CSO (PI)

CP-RND Staff

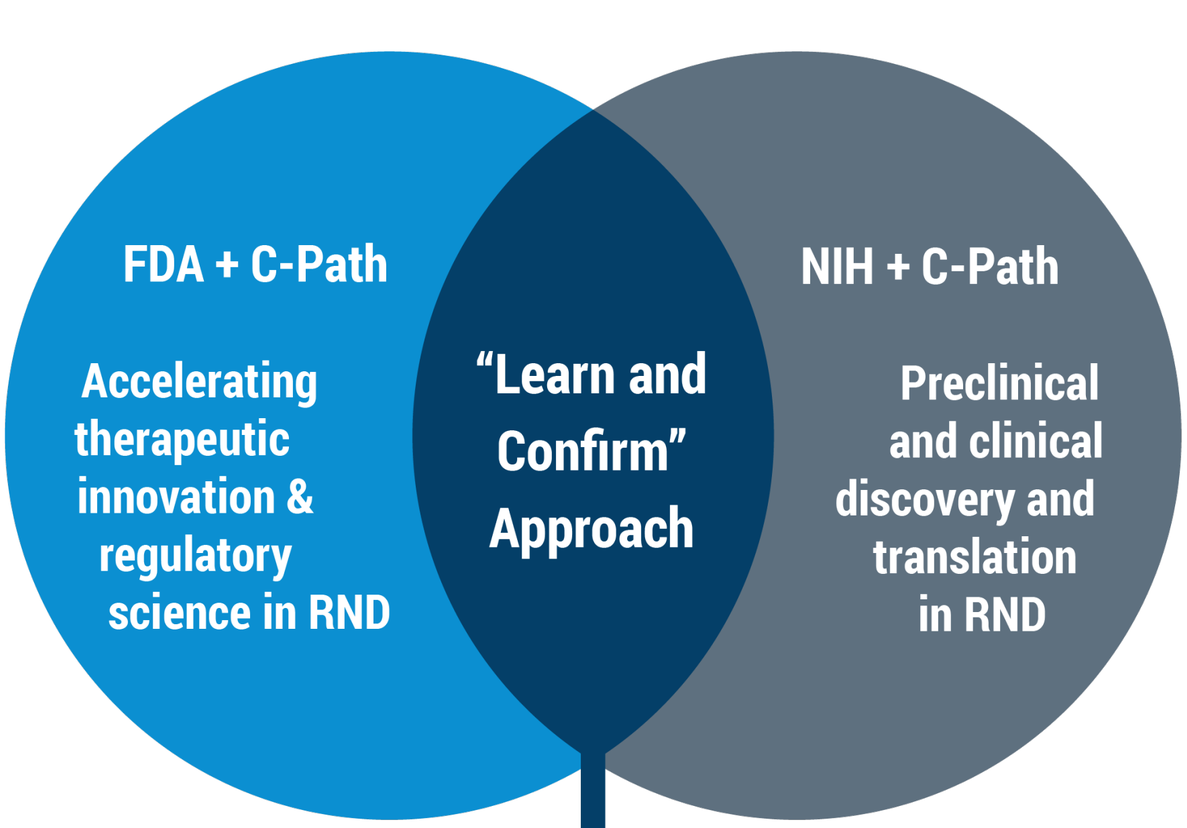
C-Path Matrix

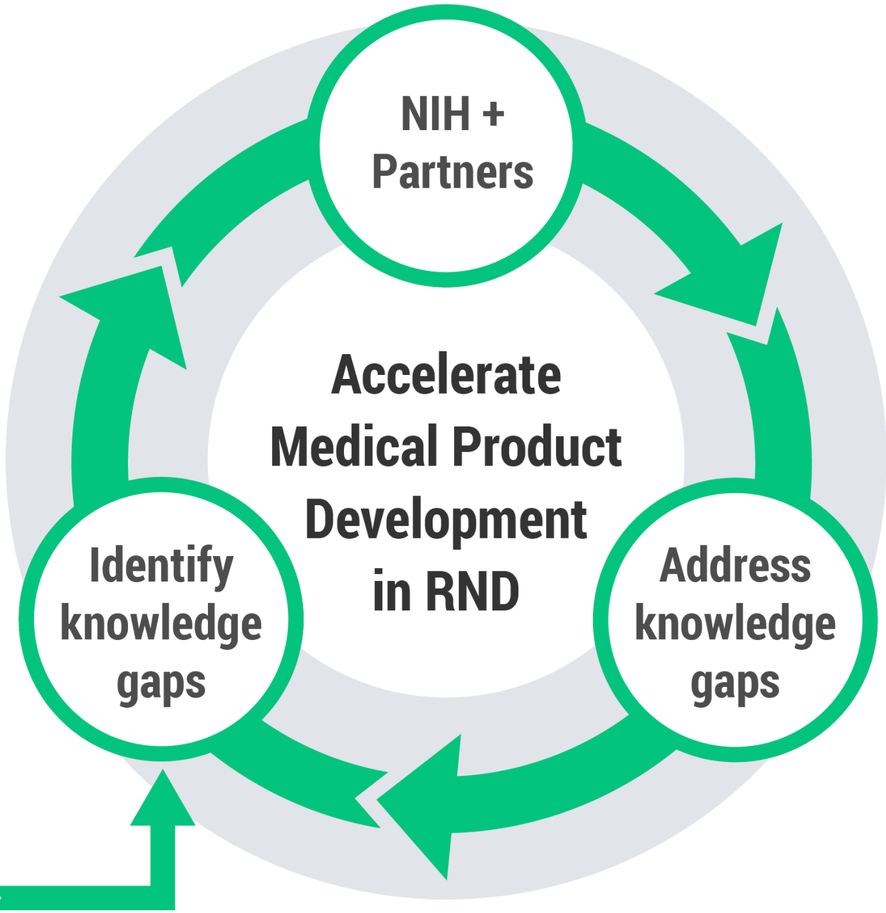
Industry POCs

Patient Advocacy POCs

Academic POCs

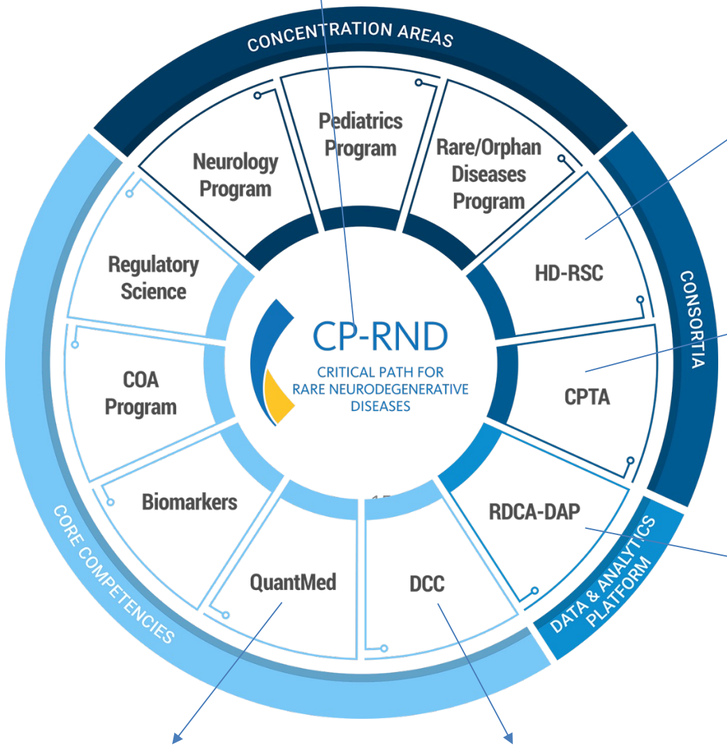
"LEARN AND CONFIRM"





UPDATES FROM C-PATH/CP-RND

ALS stakeholder and needs map under development



HD Regulatory submissions for quantitative clinical trial simulation platform to FDA and EMA 2022

Continuity for CPTA COA-focused effort, data interfacing, and disease staging definition for 2023

Defining potential regulatory strategy for FA & PSP quantitative modeling for 2023 and identifying new rare diseases for Task Groups

ALS endpoint/modeling inventory under development

Planning data strategy for ALS with NIH/FNIH

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UPDATES FOR CP-RND/NIH/FNIH/FDA

- C-Path is working with NIH and the Foundation for the National Institutes of Health (FNIH) with the intention to expand and design the Public-Private Partnership
- Planning meetings to outline an overarching ALS strategy
  - ✓ Roles and responsibilities for NIH/FNIH ALS initiative and CP-RND
  - ✓ Shared learnings
  - ✓ Data plan
  - ✓ Advocacy
  - ✓ Common roadmap and pipeline
- Next steps

HOW CAN YOU GET INVOLVED?

- We need your feedback, attend one of our upcoming open house landscape discussions
  - ✓ Patient Advocacy Forum: 3/23 2-3pm ET | 3/27 2-3pm ET
  - ✓ Industry Forum: 3/23 3-4pm ET | 3/27 3-4pm ET
  - ✓ For more information or to register email cp-rnd@c-path.org.
- Share your data
  - ✓ Clinical trials
  - ✓ Registry Data
- Attend our follow up Town Hall Meetings over the next several months and the C-Path Rare Disease Annual Meeting (September 2023)

PANEL DISCUSSION

Q&A

Place all questions in the Q&A Chat box.

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TERINA MARTINEZ, PHD

Executive Director, Critical Path for Rare Neurodegenerative Diseases and Critical Path to Therapeutics for the Ataxias

Dr. Martinez is a highly motivated neuroscientist with experience in science communication, research program management and leadership and biomarker and drug development in neurodegenerative diseases.

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MICHELLE CAMPBELL, PHD

Associate Director, Stakeholder Engagement and Clinical Outcomes, FDA

Dr. Campbell has worked on the FDA's Clinical Outcome Assessments team. Her focus is inpatient-focused drug development and the use of patient experience data in the regulatory setting.

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CELIA WITTEN, MD, PHD

Deputy Director, CBER, FDA

Dr. Witten has worked in several areas at the Food and Drug Administration, most recently as the Deputy Director at the Center for Biologics Evaluation and Research (CBER).

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WALTER KOROSHETZ, M.D.

Director, NINDS

Dr. Koroshetz has held leadership roles in a number of NIH and NINDS programs and was selected Director of the National Institute of Neurological Disorders and Stroke in 2015.

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LAUREN BOAK, PHD

Industry Co-director,
Huntington's Disease Regulatory Science Consortium (HD-RSC)

Dr. Boak has worked for Roche in both Switzerland and the United Kingdom. Her work gives her opportunities to work on potentially transformative medicines for patients with nervous system disorders.

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RONALD J. BARTEK

Co-founder/President Friedreich's
Ataxia Research Alliance

Mr. Bartek has held various voluntary positions with NORD; Alliance for a Stronger FDA, Alliance for Regenerative Medicine; NIH/NCATS; and more

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PHILIP GREEN

ALS Patient Advocate

Mr. Green was diagnosed with ALS in 2018 at the age of 48. Today, his life's work is improving the fight against this disease. He is active with many patient organizations and serves as a volunteer patient advisor to companies working on ALS therapies.

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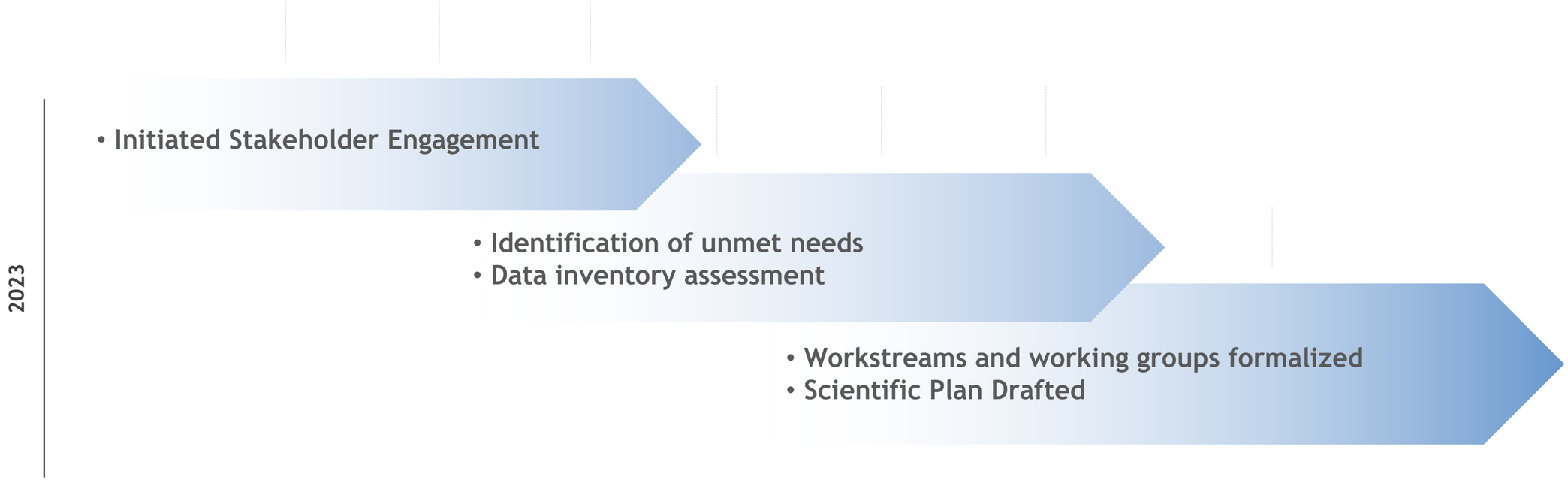
Q&A

NEXT STEPS



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Patient Introduction Webinar

March 15, 2023

2

Annual Meeting

TBD September 2023

CONTACT INFORMATION

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