Genesis of the PRO Consortium
&
Benefits of Collaboration

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FIRST ANNUAL PATIENT-REPORTED OUTCOMES (PRO) CONSORTIUM WORKSHOP

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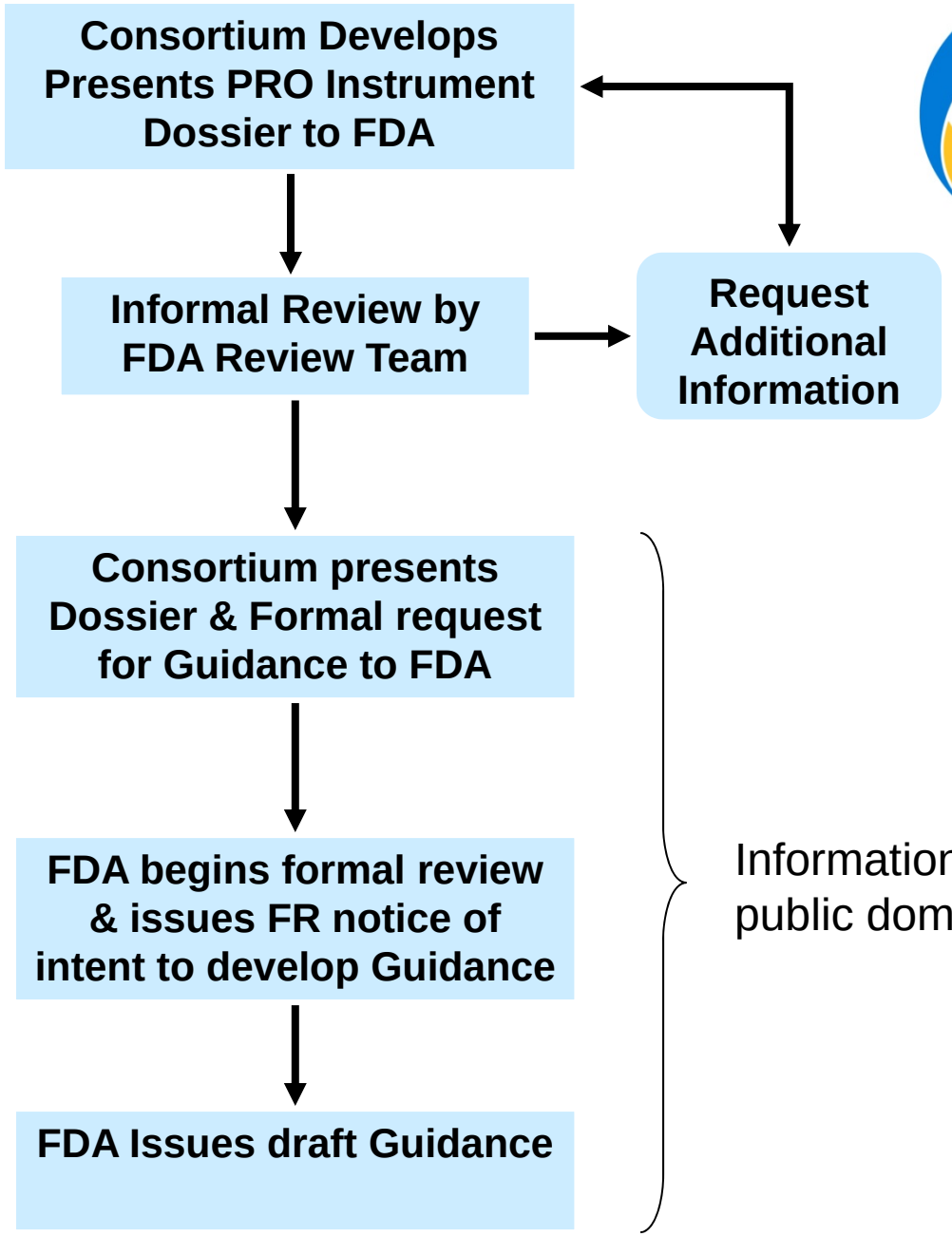
Initial Governance/Activities under PRO Consortium

- Agreement: between C-Path and members
- FDA is not a signatory
- FDA is a partner:
  - No solicitation of funds
  - Independent review of dossiers
  - Business decisions among C-Path and members are outside of FDA domain

PRO Qualification Process- *Evolving*

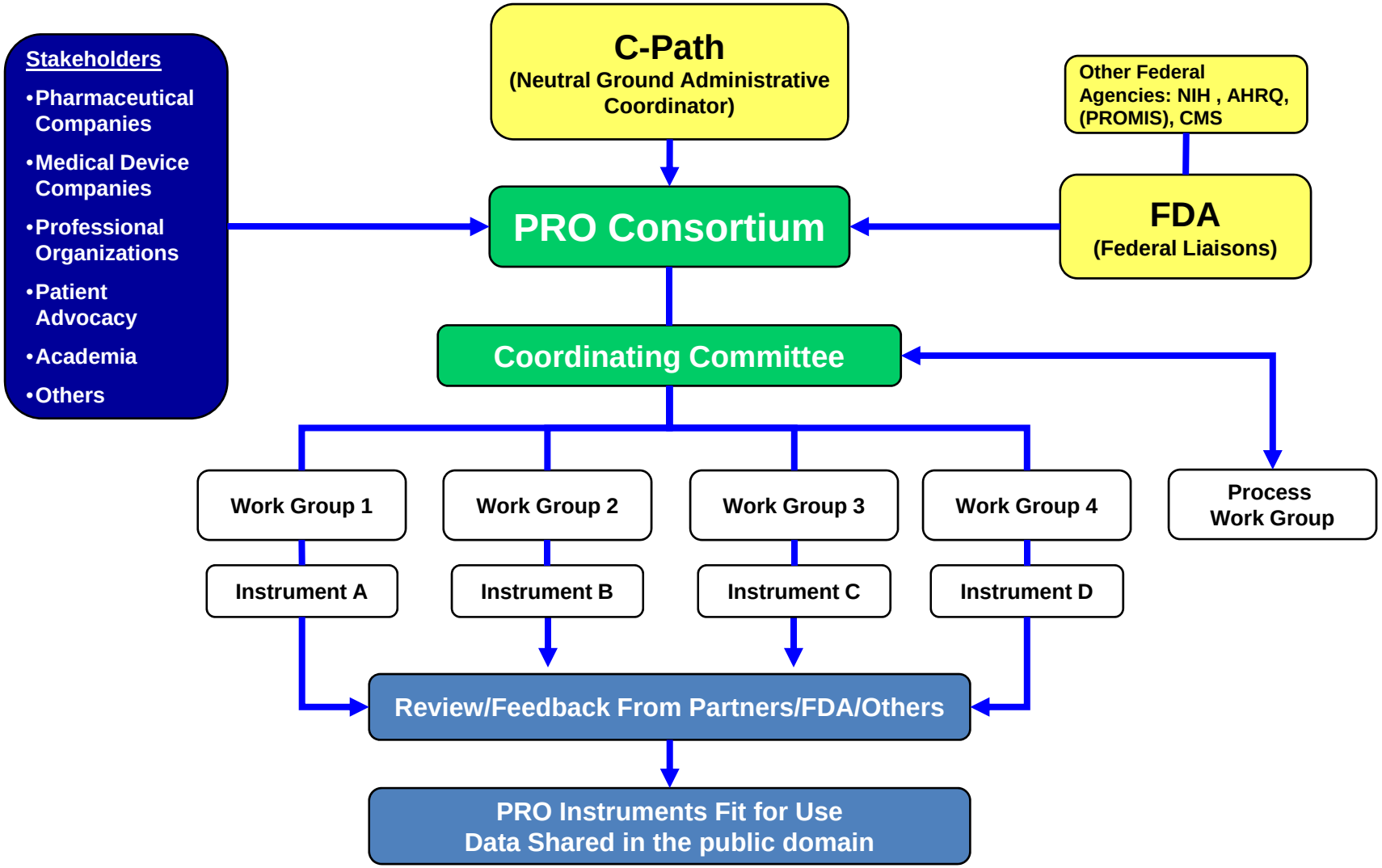
- Consortium designs (scoping documents) and conducts studies for development of instrument & supporting data for dossier *(development stage)*
- FDA provides input at ***key milestones*** and ***critical decision points***
- Dossier submitted to FDA review, with request for formal Guidance, and with summary placed in public domain by Consortium
- FDA acknowledges receipt of dossier and later issues FR notice of intent to develop Level 1 Guidance
- Formal FDA review, and development of draft Guidance as deemed appropriate
- Issue of draft Guidance


Consortium Develops Presents PRO Instrument Dossier to FDA
Informal Review by FDA Review Team
Request Additional Information
Consortium presents Dossier & Formal request for Guidance to FDA
FDA begins formal review & issues FR notice of intent to develop Guidance
FDA Issues draft Guidance
Information in public domain

Why PRO Consortium?

- Efficiency for industry/FDA: time and resources
- Standard approaches for submissions to support *(content i.e. questions)* validation and qualification *(fit for purpose…measures that support labeling claims)*
- Timely delivery of PRO instruments to patients and clinicians---and FDA

Collaboration Model



PRO Consortium Progress

- 23 member firms
- 6 disease workgroups
  - Asthma
  - Advanced Breast Cancer
  - Cognition
  - Depression
  - IBS
  - Non-Small Cell Lung Cancer
- Process workgroup
  - Staging for instrument development
  - Scoping phase summary document template for FDA submission
  - Vendor selection process (template RFP & selection criteria)
  - New workgroup proposal process
  - Guidelines for non-member participation

Benefits of This Collaboration

- Collaboration allows FDA to work with multiple partners to leverage expertise and resources toward PRO instrument development
  - Improved/transparent FDA internal (review) processes
  - Pooling of industry know-how and resources
  - Provide a basis for eventual comparison of labeling claims by physicians
  - Issuance of best practices and Guidances toward future instruments and product development

Benefits to Patients

- Content validity: evidence from patients in target population, on functionality, symptoms and disease progression…empowering patients
  - Later correlate with physician global

- Input in development of metrics to evaluate their own health status
  - understand questions/give the correct answer
  - Valid measure of a set of symptoms…correlate with outcome measures in clinical studies

Benefits to Partners

- Industry: transparency in PRO Instrument development
  - Input into concepts: test formats, methodology, nomenclature, scoring algorithm/s

- Informed product development: a well-developed PRO, developed with patient input, is enormously helpful in large trials

- Potential for applying qualified PRO instruments in multiple drug development programs

Benefits to FDA

- Build on PRO Guidance (December, 2009)
- Learn with the development of each PRO Instrument developed
- Consistency of submission…easy during review/endpoint assessment
- Inform the development of future instruments & Guidances
- Harmonization with international regulators: EMA

PPP Value Assessment

- FDA partnering with U Maryland to develop metric s to assess performance and outcomes PPP programs…value to stakeholders and patients

- PRO consortium will be evaluated as case study: What's working/what's not, how can we improve

- Ritu Agarwal & Kenyon Crowley, Director and Associate Director, Center for Healthcare Information and Decision Systems

Balanced Scorecard