Drug Development Tools for Kidney Disease

The Nuts and Bolts of Forming a Consortium

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10 YEARS

Steve Broadbent
Critical Path Institute

Value of Consortium Model

DDT-KD Consortium

- Accelerates the ability to learn and answer critical research questions through the sharing of experience, knowledge, and data
- Spreads the cost and risk to advance research in areas of unmet need
- Engagement of regulatory authorities helps assure deliverables will be accepted in regulatory process

Kidney Disease Consortium

DDT-KD Consortium

Establish a new consortium to support collaborative research and regulatory endorsement of new, effective methods for evaluating the safety and efficacy of therapies in chronic kidney disease and acute kidney injury

Initial Objectives:

- Create a database of aggregated, standardized clinical data to support regulatory submissions and research. Update CDISC standards as needed.
- Create scientific consensus on the optimal prognostic biomarkers for use in clinical trials for therapies to treat CKD and AKI that can be advanced for regulatory endorsement by the FDA and EMA

Potential Objectives:

- Identify and qualify diagnostic, predictive, or pharmaco-dynamic biomarkers
- Develop a disease progression model and simulation platform to inform clinical trial design
- Other clinical outcome assessment methods

Membership Legal Agreement

DDT-KD Consortium

- Initial Scope
- Responsibilities and Expectations of Members
- Governance
- Confidentiality
- Intellectual Property
- Publications and Publicity
- Fees
- Anti-Trust
- Anti-Corruption, Anti-Bribery
- Termination, Liability, Indemnification, etc.

Governance Models

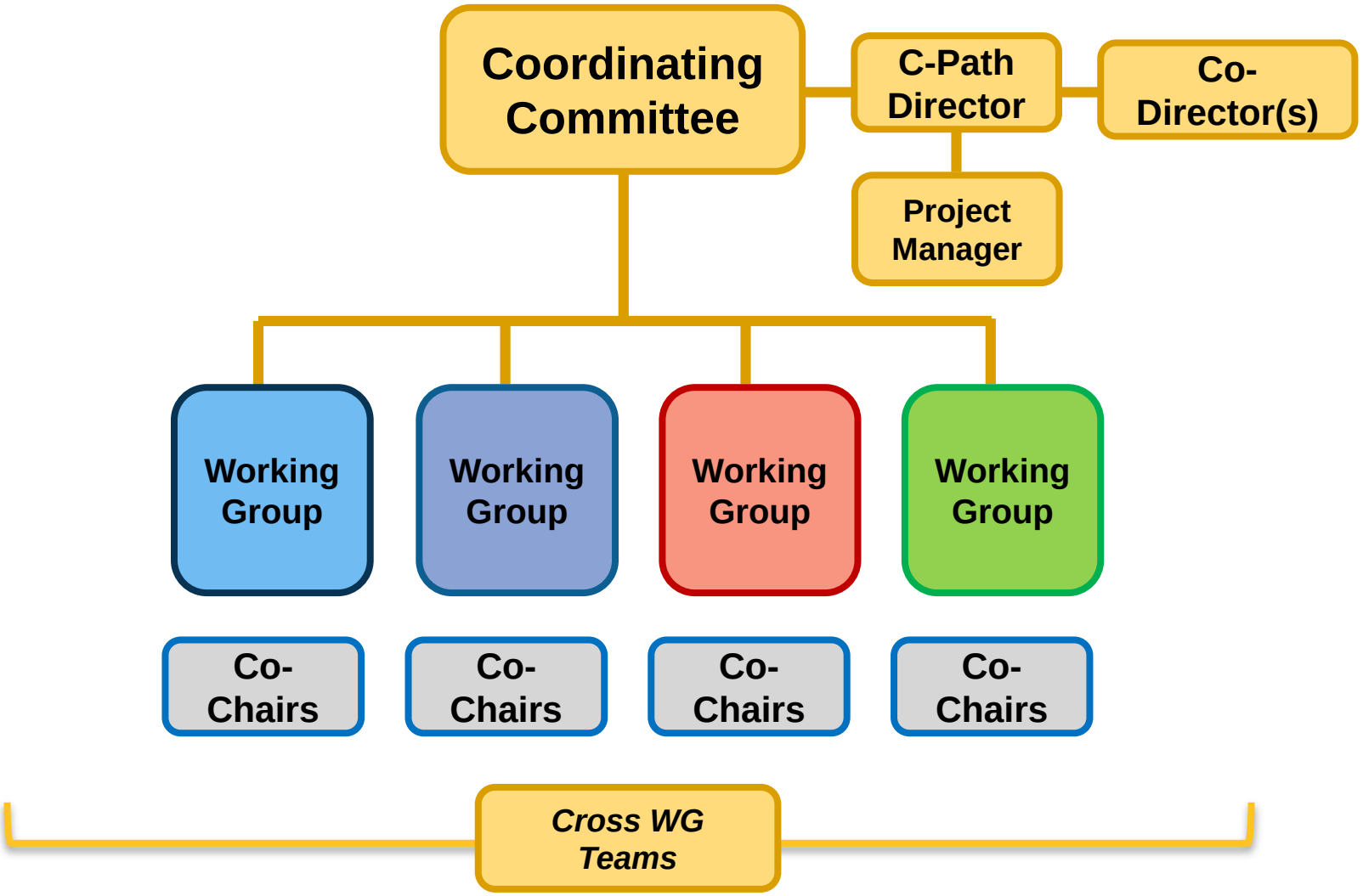
DDT-KD Consortium

Project governance to be informed by C-Path best practices

- Leadership Team consisting of C-Path executive director and founding partner co-director(s) as primary leadership team
- Coordinating committee with representation for all members makes all significant decisions
- Separate Working Groups created to focus on each deliverable – led by a chair or co-chairs

Typical Governance Structure

DDT-KD Consortium



Data Capability & Safeguards

DDT-KD Consortium

Establish a pooled, standardized, secure database of clinical trial data

- Data access is determined by owners/contributors of the data
- Full data de-identification that meets HIPAA “Safe Harbor” specifications
- C-Path CODR database platform
  - Extensive security measures for online data access & database management
  - Proven database technology
- Leverage existing data standards partnerships
  - C-Path consortia expertise
  - CFAST data standards project with CDISC

Clinical Data Shared with C-Path

DDT-KD Consortium

Consortium	Therapeutic Area	# of Studies	Total Number of Subjects	Range of Subjects per Study		Duration (wks)		Number of Data Contributors
				Min	Max	Min	Max	
Coalition Against Major Diseases	Alzheimer's disease	32	12,960	57	1581	12	156	12
	Parkinson's disease	9	5069	80	1174	40	147	3
Critical Path to TB drug Regimes	Tuberculosis	14	4492	68	1075	1	24	5
MS Outcome Assessments Consortium	Multiple sclerosis	16	14,432	239	1515	9	234	9
Polycystic Kidney Disease	Polycystic kidney disease	5	2941	202	1112	1yr	67 yrs	4
Predictive Safety Testing Consortium	Normal healthy volunteer-kidney	1	172	172	172	1 day	7	1

77 Studies **40,066 Subjects** **34 Contributors**

Project Management

DDT-KD Consortium

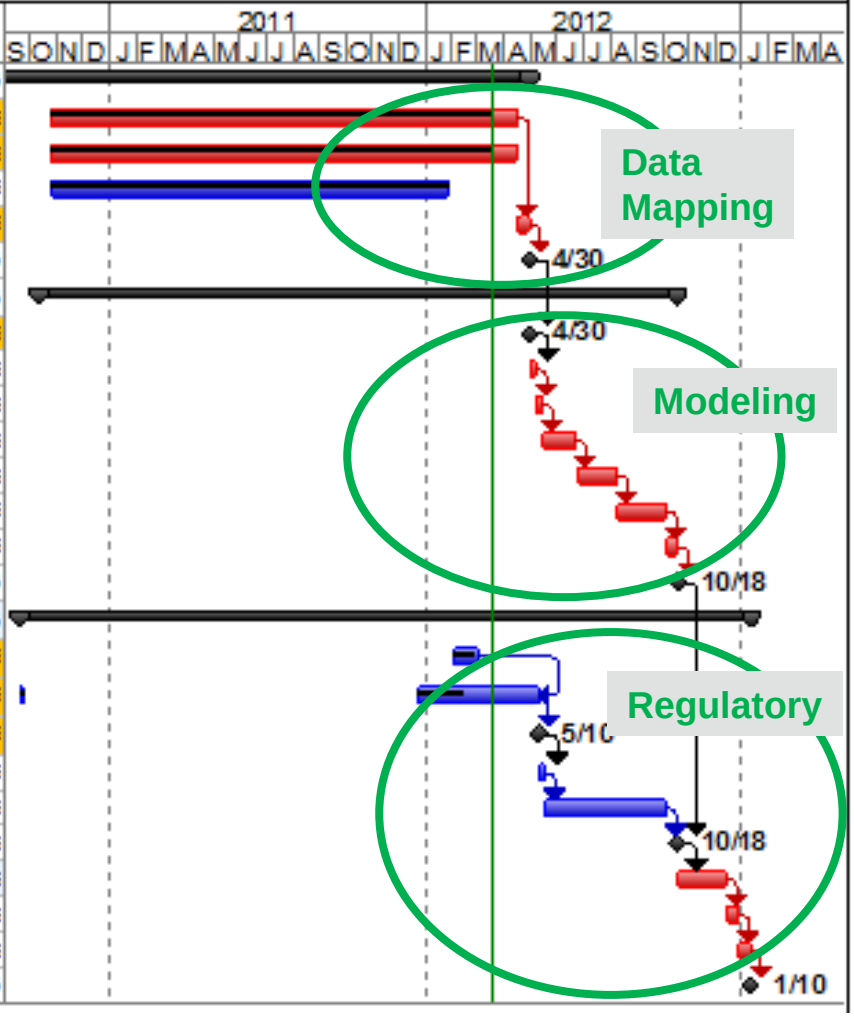
- Written Goals and Deliverables
- Project Plan with Schedules
- Clear Tasks with Owners
- Tracking and Communicating
- Budgets and Finance
- Meetings and Workshops

Typical Project Schedule

DDT-KD Consortium

PKD Outcome Consortium — 3/20/12

ID	Task Name	Duration
38	**Map clinical trial data and load database**	**522 days**
54	Map Mayo Data	386 days
55	Map Emory Data	386 days
56	Map U Colorado Data	330 days
58	Upload/VerifyMayo, Emory, Colorado Data	10 days
62	**Data Loaded/Verified In the Database**	**0 days**
64	**Disease Modeling and Simulation**	**528 days**
71	Initiate Modeling and Analysis Phase	0 days
72	Aim 1: Modeling and Simulations Plan	5 days
73	Aim 2: Briefing Package Review	5 days
74	Aim 3: Disease Progression Model	29 days
75	Aim 4: TKV Expansion and Clinical Outcomes	34 days
76	Aim 5: Biomarker Qualification Package	40 days
77	Disease Modeling Results and Review	10 days
78	**Disease Modeling Complete**	**0 days**
80	**Regulatory Qualification Process**	**604 days**
90	BQRT Briefing Book Recommendations	21 days
91	Update BQS Briefing Package	104 days
92	Submit Updated BQS Briefing Package to FDA	0 days
93	Conduct initial BQRT review of Briefing Pkg	5 days
94	Consultation and Advice Phase activities	100 days
95	FDA agreement to proceed to Review Phase	0 days
96	Prepare Final Qualification Package	40 days
97	Internal Review of Final Qualification Package	10 days
98	Finalize Qualification Package	10 days
99	RegulatorySubmission Complete	0 days



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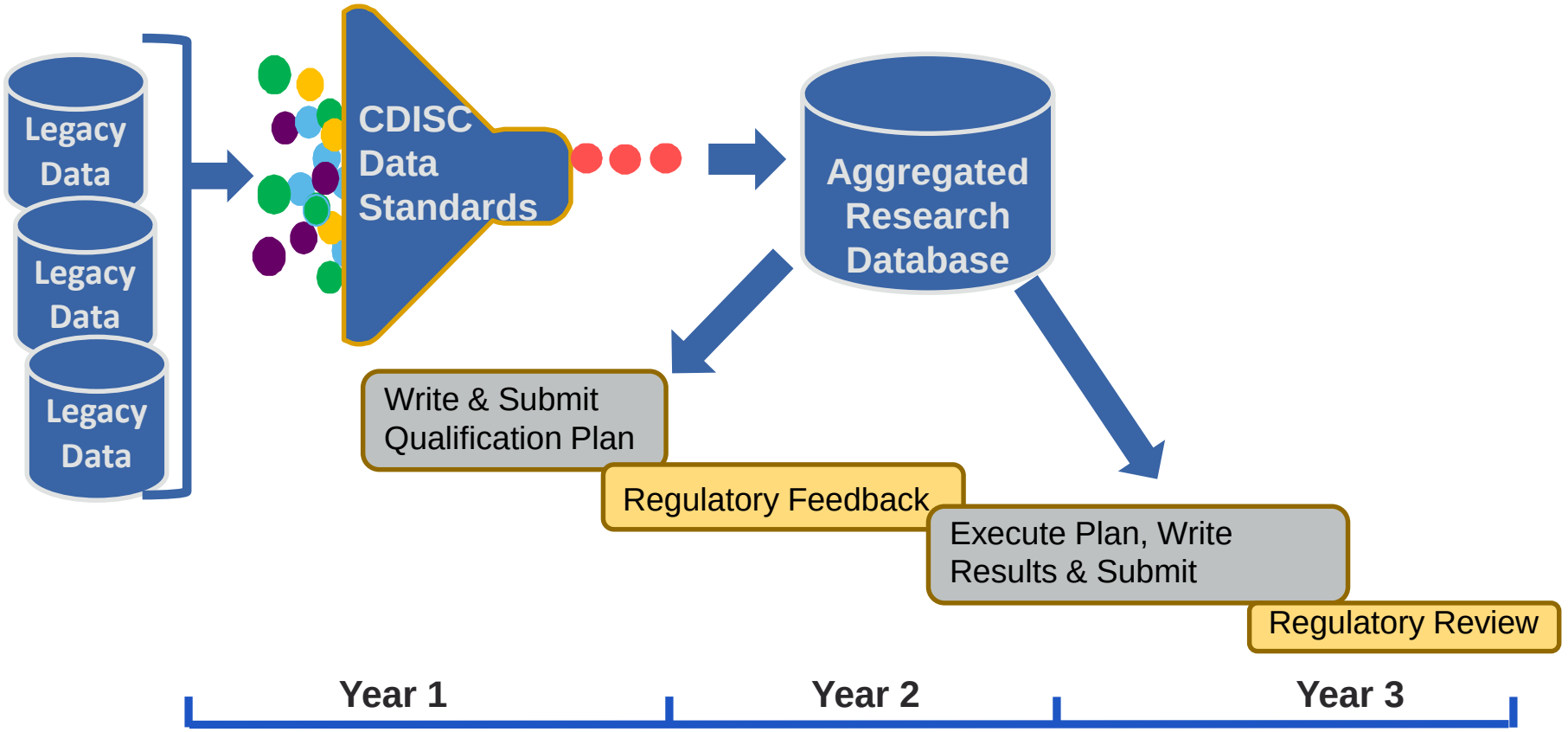
Proposal Scope and Timeline

DDT-KD Consortium

- Development of a data sharing platform for clinical data
- Complete/Update CDISC therapeutic area standard where gaps exist
- Use data to inform the development of regulatory documents and publications



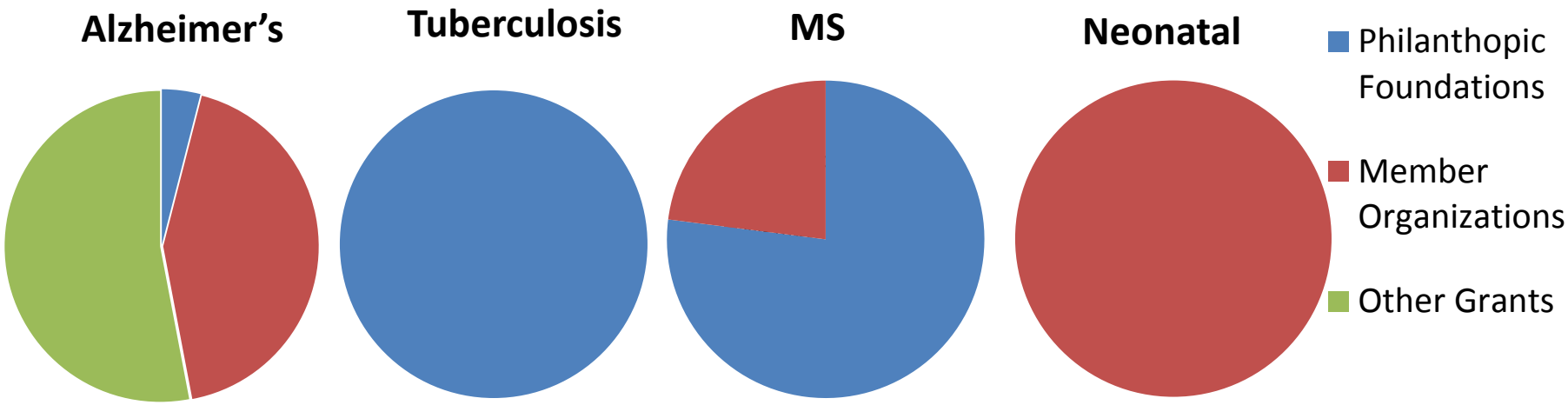
Funding Models

DDT-KD Consortium

Funding could be provided through multiple sources:

- Philanthropic foundations
- Member organizations
- Other grants
- Combination of one or more of the above

C-Path funding model examples:



Next Steps

DDT-KD Consortium

- Review proposal with all potential founding partners
- Refine Project Proposal as needed
- Finalize consortium membership agreement
- Announce and formal launch
- Staff working groups and select leadership
- Ramp up to full scope once sufficient organizations have agreed to join consortium and required funding level is achieved