

First Day Overview

Dr. Hamburg:

Important: collaboration & standard setting

Challenges: 1) Coordinate, 2) Prioritize, 3)
Find incentives for academics to develop
focus on regulatory science

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Data Standards

Challenge of the Yottobite – 4 mile high
tower of iPads

PDUFA V : data standards are a priority

55n5 – The next frontier: from genome to
proteome to “disease-ome”

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Scope of work suggestion: Standardize data needed for 1° and 2 ° endpoints

Should include special populations in standards

Need to link to molecular medicine – standards for reports of genetic tests.

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Biomarkers

Importance of consortia to maximize value and share burden

Prioritize biomarkers that improve public health

Qualification process should be nimble and efficient because science moves fast

Need to anticipate need so that the very best tools are available when needed

(using the wrong tool can generate bad data)

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Biomarkers

Disease Progression BM may not respond as expected to an intervention

Are there Venture Philanthropy opportunities

YES!!!!!!!!!!!!!!!!!!!!

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PROs

25-30% of labels include PRO claims – likely to grow rapidly - PCORI

PRO consortium C-Path's largest with 24 members

PRO, ClinRO and ObsRO

ePRO consortium for electronic platforms

Content Validity must be established early

Need to communicate to small companies

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Disease Progression Models

Models can help assess the relative value of a new drug candidate

Can explain why drugs might have failed

Can be used to do virtual experiments, e.g. delayed start design trials

Enable subset analyses

Used to evaluate or “qualify” biomarkers

MCI models – great value from biomarkers

Tomorrow

Breakfast 7:30 – 8:30

Breakout groups on Consortia 8:30 -10AM

Thank you: Dr. Michel Goldman, IMI
Senator Bob Bennett,
Kim Bourne,
Barrett Stradford –
Critical Path Foundation
Students VIDEO

Thank you for your participation

