Session Background

Patient-centric designs: Involving patients and families

Simon Day, PhD

simon.day@CTCT-Ltd.co.uk

CTCT Clinical Trials Consulting & Training Limited

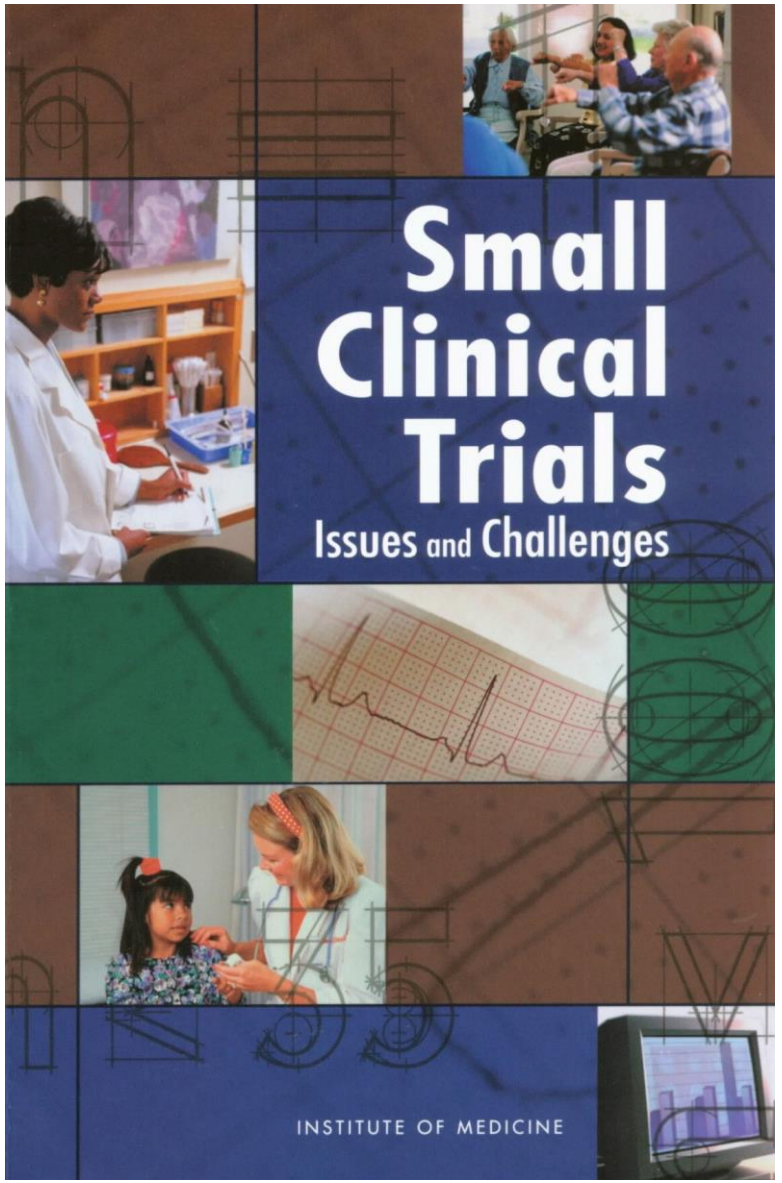
Where is this session coming from? And where is it going?

- We used to have physicians and statisticians design clinical trials

- But then we learnt!

Some beginnings

Institute of Medicine, 2001



My beginnings…
EU Regulatory Guidance

European Medicines Agency

London, 27 July 2006
Doc. Ref. CHMP/EWP/83561/2005

COMMITTEE FOR MEDICINAL PRODUCTS FOR HUMAN USE (CHMP)

GUIDELINE ON CLINICAL TRIALS IN SMALL POPULATIONS

DRAFT AGREED BY EFFICACY WORKING PARTY / AD HOC GROUP ON CLINICAL TRIALS IN SMALL POPULATIONS	May 2002 – January 2005
ADOPTION BY CHMP FOR RELEASE FOR CONSULTATION	March 2005
END OF CONSULTATION (DEADLINE FOR COMMENTS)	September 2005
AGREED BY EFFICACY WORKING PARTY	July 2006
ADOPTION BY CHMP	27 July 2006
DATE FOR COMING INTO EFFECT	1 February 2007

Regulatory Guidance

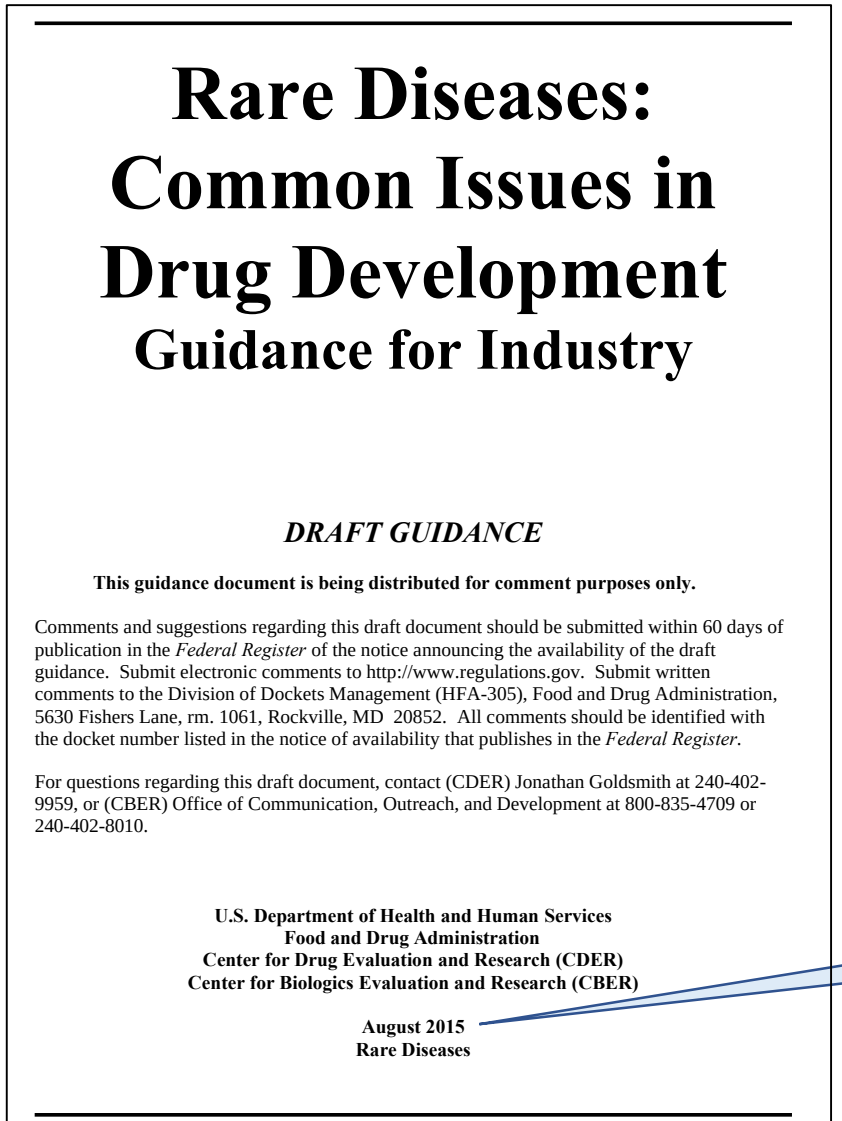
- Almost nothing at the time (early 2002)

- Tension about “upholding standards”
- “Nothing should contradict other guidance”

and

- Need for pragmatism, flexibility, etc.

and US Regulatory Guidance



Rare Diseases: Common Issues in Drug Development

Guidance for Industry

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 60 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to http://www.regulations.gov. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document, contact (CDER) Jonathan Goldsmith at 240-402-9959, or (CBER) Office of Communication, Outreach, and Development at 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

August 2015
Rare Diseases

Also January 2019

Working Groups (and similar)

Three major EU funded projects

- InSPiRe
- Innovative methodology for Small Populations Research

  http://www.warwick.ac.uk/inspire/

- Asterix
- Advances in Small Trials dEsign for Regulatory Innovation and eXcellence

  http://www.asterix-fp7.eu/

- IDeAl
- Integrated Design and Analysis of small population group trials

  http://www.ideal.rwth-aachen.de/

Working Groups (and similar)

- IRDiRC first goals (2011)
  - 200 new therapies by 2020

Working Groups (and similar)

IRDiRC
INTERNATIONAL
RARE DISEASES RESEARCH
CONSORTIUM

- IRDiRC first goals (2011)
  - 200 new therapies by 2020

Working Groups (and similar)

IRDiRC
INTERNATIONAL
RARE DISEASES RESEARCH
CONSORTIUM

- IRDiRC first goals (2011)
  - 200 new therapies by 2020

- IRDiRC new goals (2017 – 2027)
  - 1000 new therapies for rare diseases will be approved, the majority of which will focus on diseases without approved options

Working Groups (and similar)
“Small Population Clinical Trials” Task Force

Lots of good stuff, but still…

- "Investigators have told us that patients would be unwilling to enter a placebo-controlled trial"

- "The sponsor believes it is not ethical to randomize patients in a trial lasting longer than 6 months"

Still not so much about:

"we have asked patients and they have told us…"

Lots of good stuff, but still…

- Examples like this led us to start to collect short “case studies”
  (we all learn from our mistakes!)

If you have any good, illustrative, examples to share…
please contact the session Chairs (or me)

To close… and to lead on…

- The purpose of this session is to illustrate what patients, parents, carers, can bring to the discussion
- How best to get them involved… and when
- Examples from trialists who have done this, successfully!