

EuroWilson:

the first prospective Europe-wide study of Wilson's Disease

challenges, difficulties, solutions → success

Issues

- 1.Purpose
- 2.Register or database?
- 3.Case definition
- 4.Database content
- 5.Terms and language
- 6.Data quality and cleaning
- 7.Clinician co-operation
- 8.Team working
- 9.Consent & governance
- 10.Research ethics committees
- 11.Data handling
- 12.Access to the data
- 13.Sustainability

1. Purpose

To assess the feasibility of clinical trials in Wilson's

Mission creep

additional aims

RISK =
loss of focus

- "Whilst we are about it" *eg epidemiology*
- Quality *eg EMQN*
- Unexpected findings *vertical transmission*
- "a pity to stop"

2. Register or database?

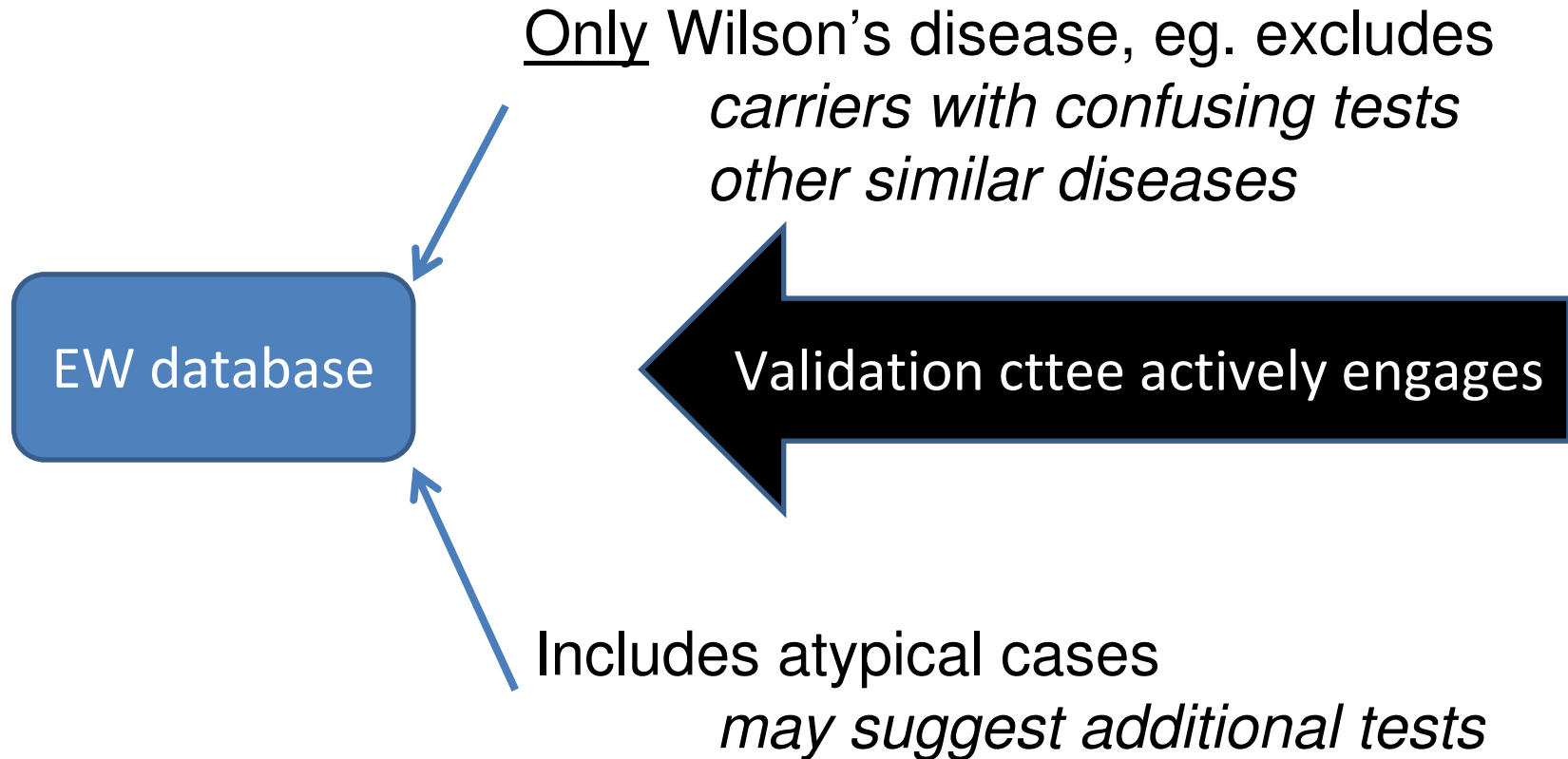
EW is a clinical database of patients defined as having Wilson's disease

Attempts complete ascertainment, but achieves its objectives without it

A register requires that all patients with [X] are notified to it

e.g. statutory, mandatory, treatment related

3. Case definition



4. Database content

“Let’s include everything in case we need it later”

“We must not over-burden the data-entering clinicians”

EW database

Core
mandatory

+

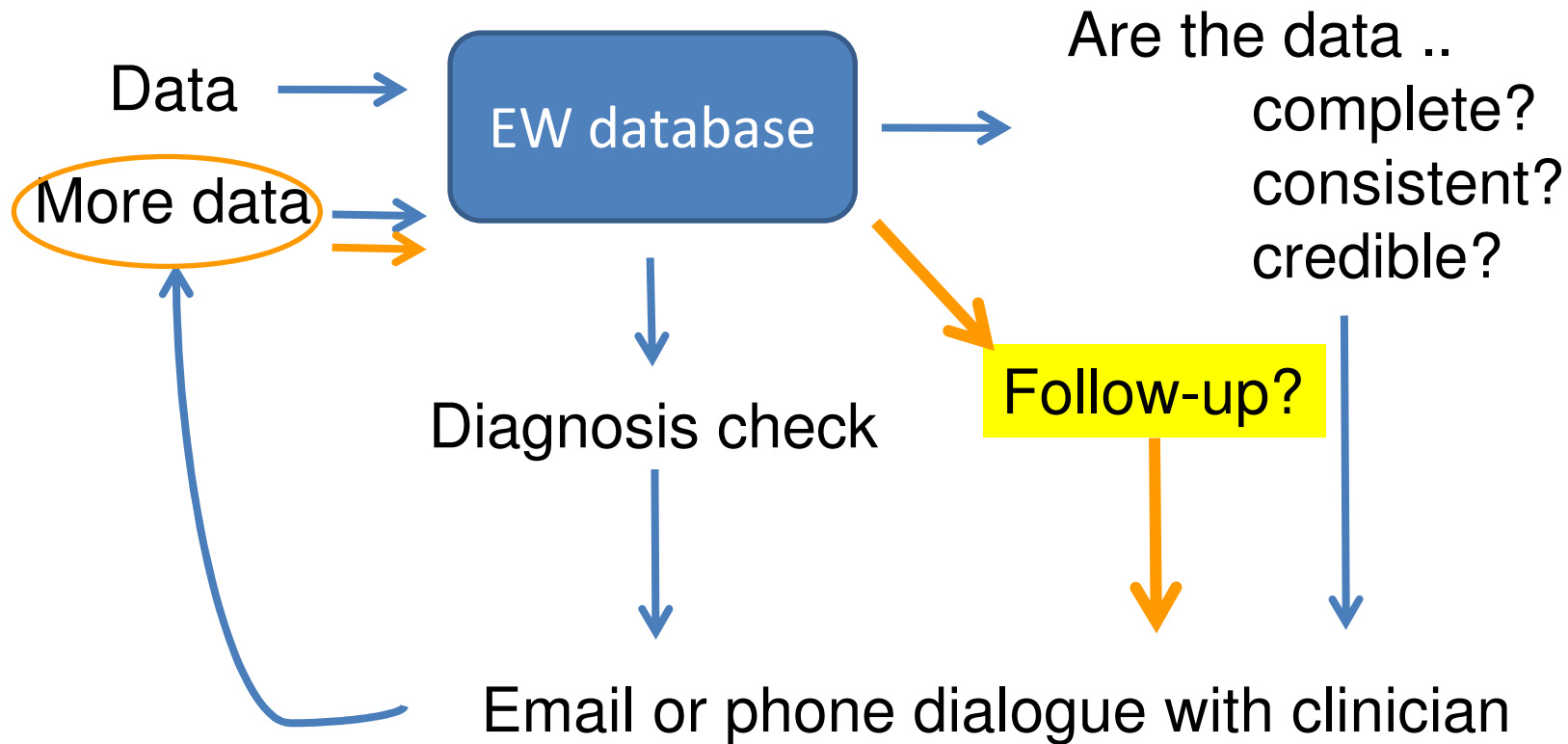
Detailed
desired

5. Terms & language

A group of English-speaking doctors thought everybody knew the meaning of words like

.... asymptomatic
..... presentation
..... proband
..... normal

6. Data quality & cleaning



7. Clinician co-operation



is an academically-led



project

So, data entry by a clinician:

1. Carries no financial reward
2. Does not confer formal specialist status upon the centre
3. Relies on goodwill, friendship, and Hippocratic principles
4. Does lead to names on publications & education



Relative merits of this vs

1. Heavily funded industry-led databases
2. Statutory registers

8. Team working



Paediatrician

"I don't know what happens after they get to 16"



Adult hepatologist

"I can fix the liver, but please don't ask me to do a neurological examination"

Neurologist

The liver?



ophthalmologists
psychiatrists
rheumatologists
haematologists

"I had a case once"

9. Consent & governance

Is my data safe?

- see www.eurowilson.org for information & consent forms for adults, children, & parents
- We regard patient data as **a conditional gift**, so
 1. Ownership of data passes from donor to Consortium
 2. The conditions of the gift are explicit in the consent form eg confidentiality, security, use of data
- An Oversight Committee with patient representatives and ethics expertise ensures probity

10. Research ethics committees

- REC approval initially in Coordinator's country (UK)
- MREC imposed stifling condition that each data-entering clinician had LREC approval; appealed
- REC requirements varied hugely in participating countries; usually accepted version of UK MREC forms
- Concerns about cross-boundary flow of data

Suggestion:

A European REC approval system for rare disease registers, agreed by all MS, would reduce delays and inconsistencies and increase rigour.

11. Data handling



uses an anonymised web-based system with CPS card security for entry and secure systems for storage and back-up



doctor

- mutual ignorance
- & lack of esteem
- different languages

Unexpectedly hard work to achieve and maintain dialogue



techie

12. Access to the data

1. The data is owned by the Consortium
2. Applications for use, whether academic or commercial, are made to the Consortium (may refer to Oversight)
3. Permission for use should not be unreasonably with-held
4. An executive summary for public use is a condition of the FP6 grant
5. Publication rules laid down at the beginning in a binding Consortium Agreement

13. Sustainability

Having achieved its immediate goals, could be “mothballed,” **BUT**

The logo for EuroWilson, featuring the text "EuroWilson" in white on a dark purple background. To the right of the text is a stylized graphic of a yellow and white swoosh with horizontal lines, suggesting a wing or a signal.

EuroWilson

Important new answerable questions have arisen
It is a unique resource and exemplar

Suggestion

Maintenance of RD databases:

1. Should not depend on ad hoc funding
2. If coordinated at EU level, could achieve economies of effort and scale

Thanks to



- the patients
- Dr Catherine Berens
- the EuroWilson Consortium,
Oversight Committee,
staff, and
data-entering clinicians
- and, if you have been, to you for listening