

The Asterix project

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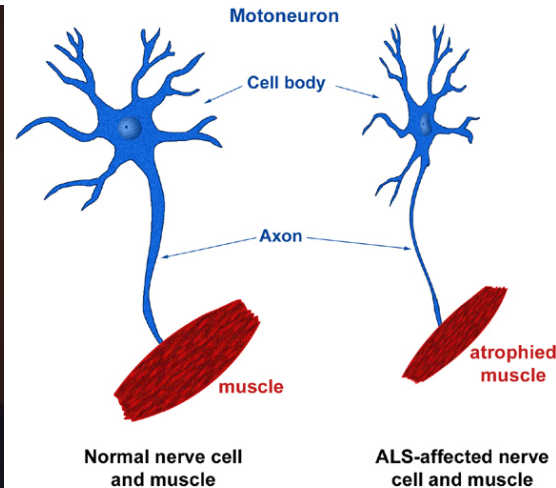
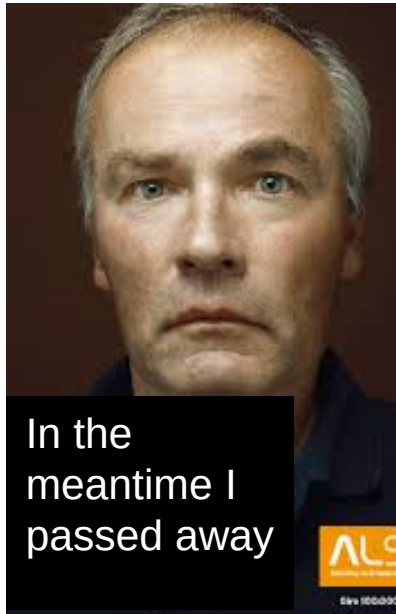


Asterix



- Concept and objectives
- Key progress and output to date
- What is still ahead of us?
- Towards guidance for practice

Concept and objectives: Patients



International groundbreaking genetic ALS research

To understand the genetic basis of ALS, we want to map the DNA profiles of 15,000 ALS patients. To make Project Mine work, we need your help. Make it yours!

goal
15,000 DNA profiles

14%
raised

collected so far
2,069.56 DNA profiles

We want to map the DNA profiles of 15,000 ALS patients.

Will you help us reach our goal?

Concept and objectives: Call



HEALTH.2013.4.2-3: New methodologies for clinical trials for small population groups. FP7-HEALTH-2013-INNOVATION-1. The objective is to develop new or improved statistical design methodologies for clinical trials aiming at the efficient assessment of the safety and/or efficacy of a treatment for small population groups in particular for rare diseases or personalised (stratified or individualised) medicine. Research should be multidisciplinary and should involve all relevant stakeholders including industry and patient advocacy groups as appropriate. Ideally, results would lead to improvement of clinical trial guidelines. Clinical trials as such are excluded from this topic. Collaboration with relevant organisations outside Europe is welcomed.

Note: Limits on the EU financial contribution will apply and will be implemented strictly as eligibility criterion.

Funding scheme: Collaborative Project (small-scale focused research project).

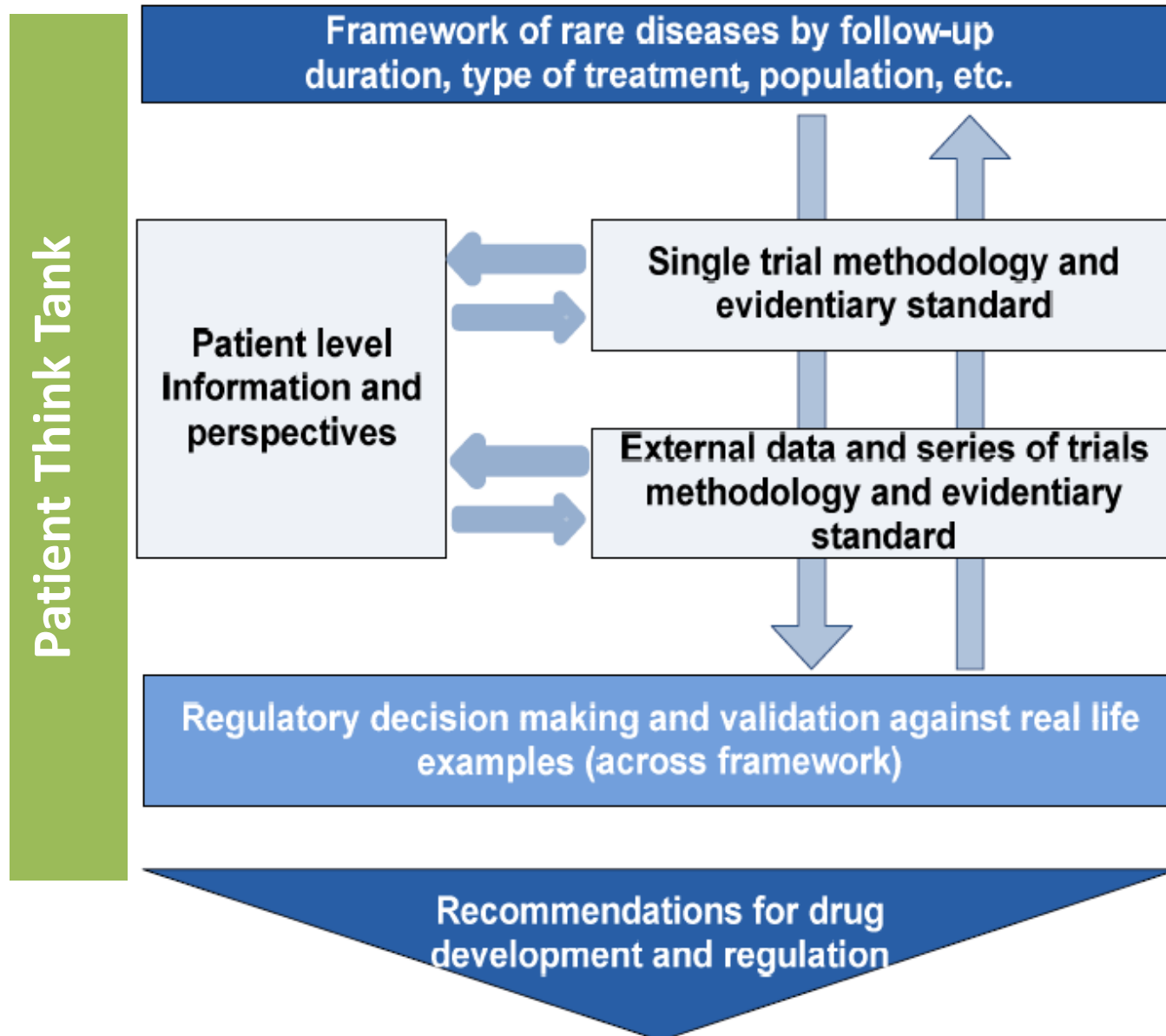
One or more proposals may be selected.

Expected Impact: Cost efficient clinical trials deriving reliable results from trials in small population groups.

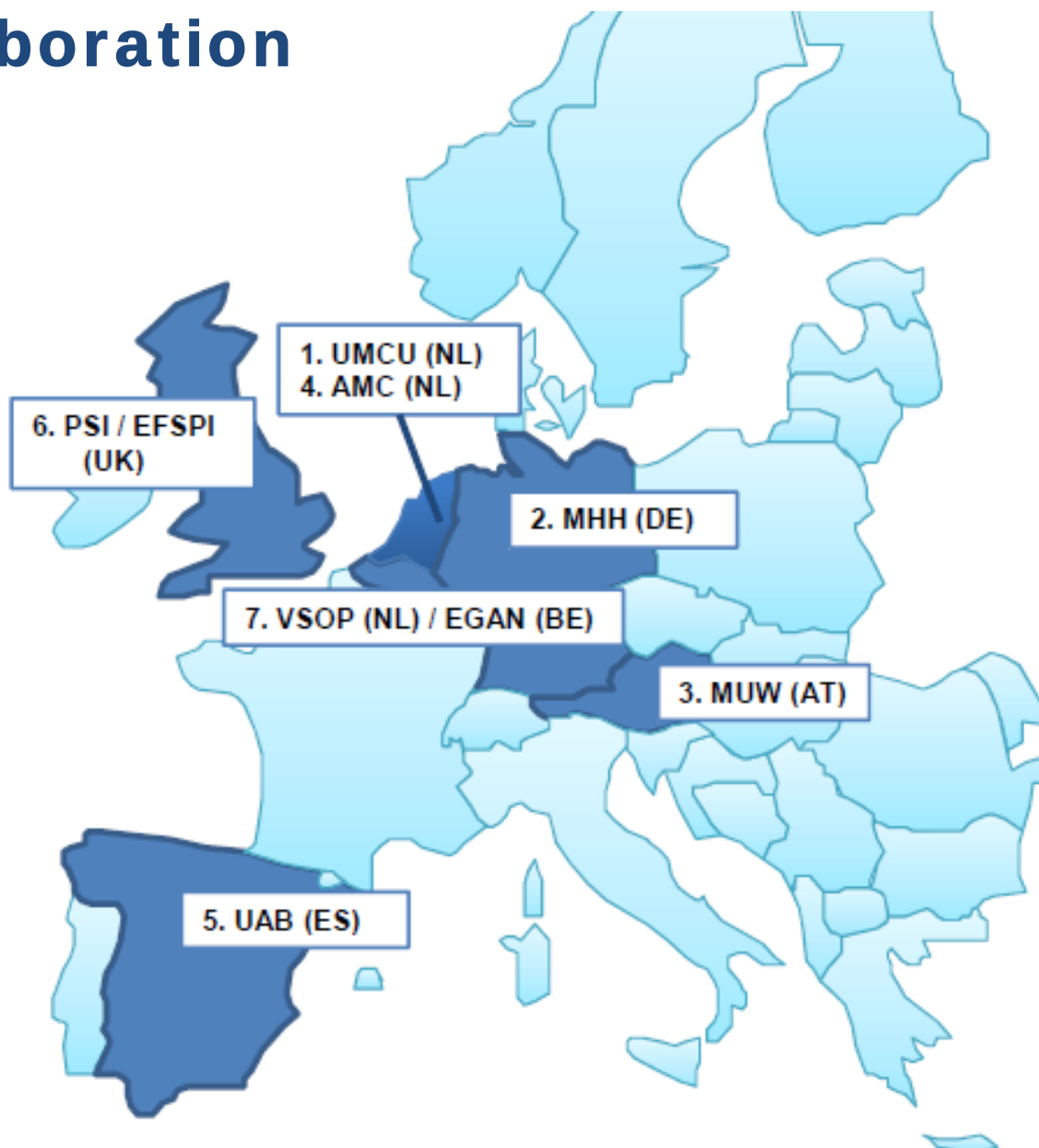
Additional eligibility criterion:

The requested EU contribution per project shall not exceed EUR 3 000 000.

Concept and objectives



Collaboration



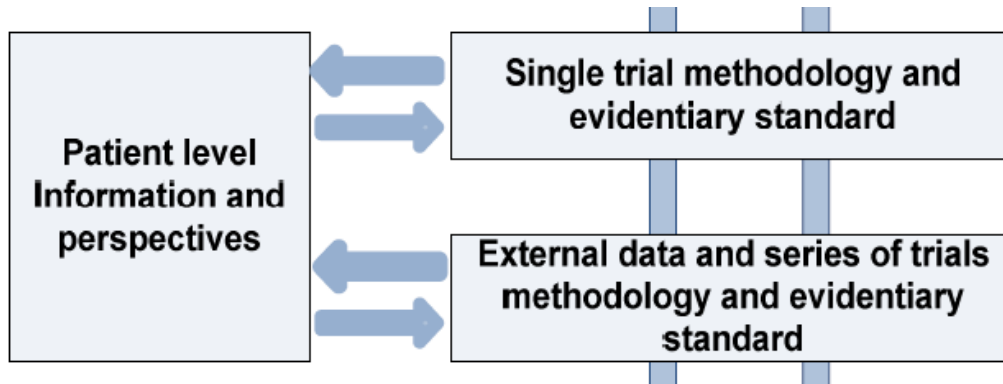
Concept and objectives



Unique features in project execution

- Included developments beyond statistical methodology
 - Explore new clinically relevant endpoints
 - Ethical framework
 - Disease/condition framework
 - Models for patient involvement
- Regular interaction between patient representatives and researchers.
- Strong focus on PhD projects: educate and train the next generation of clinical trial statisticians/methodologist.

Key progress and output



Some highlights:

- Stratification in small population trials
- Goal Attainment Scaling
- Sequential and adaptive trials in small populations
- Multiple endpoints in small samples
- Meta-analysis and borrowing of information (in sparse settings)
-

Key progress and output

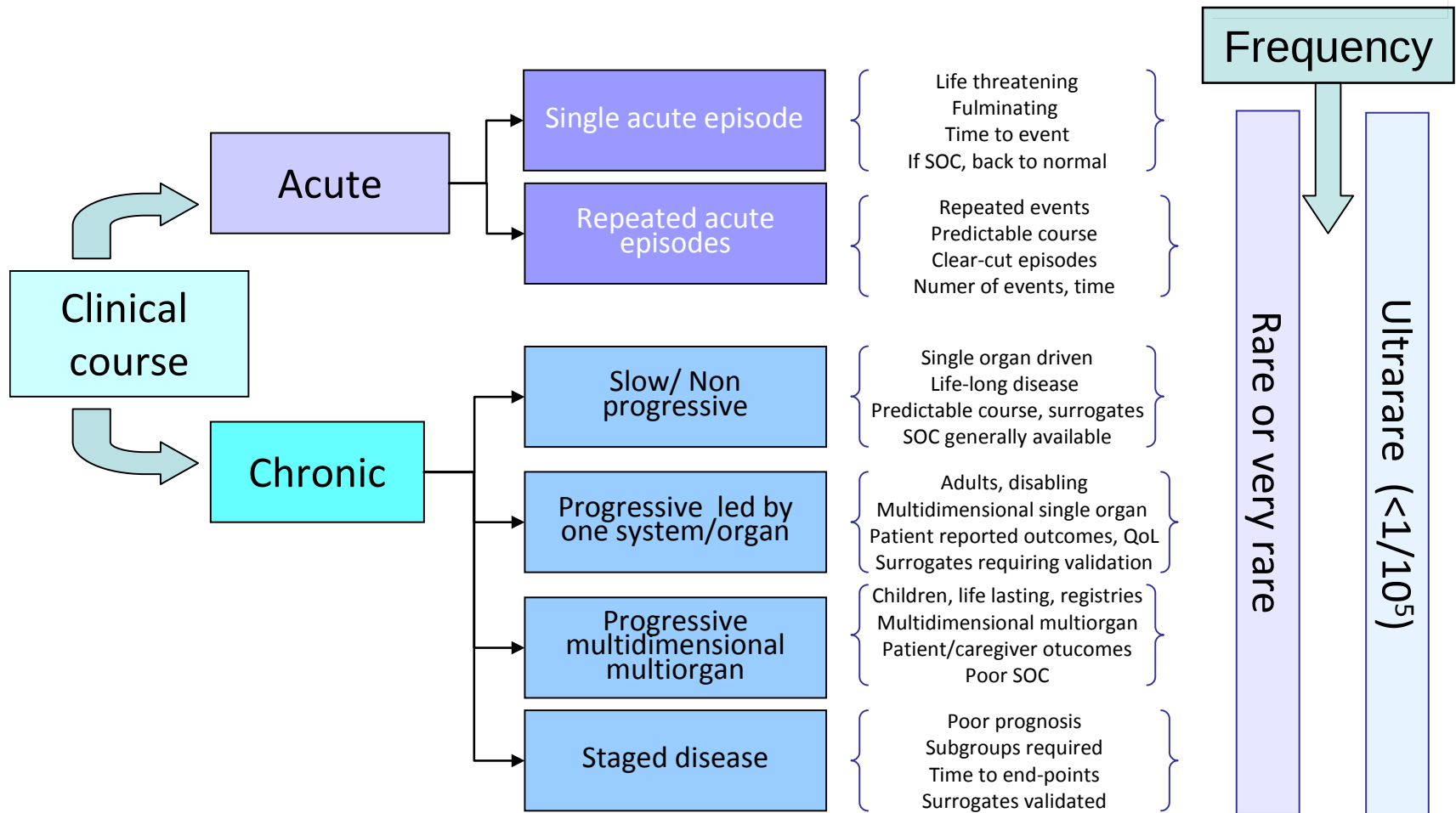


Framework of rare diseases by follow-up
duration, type of treatment, population, etc.

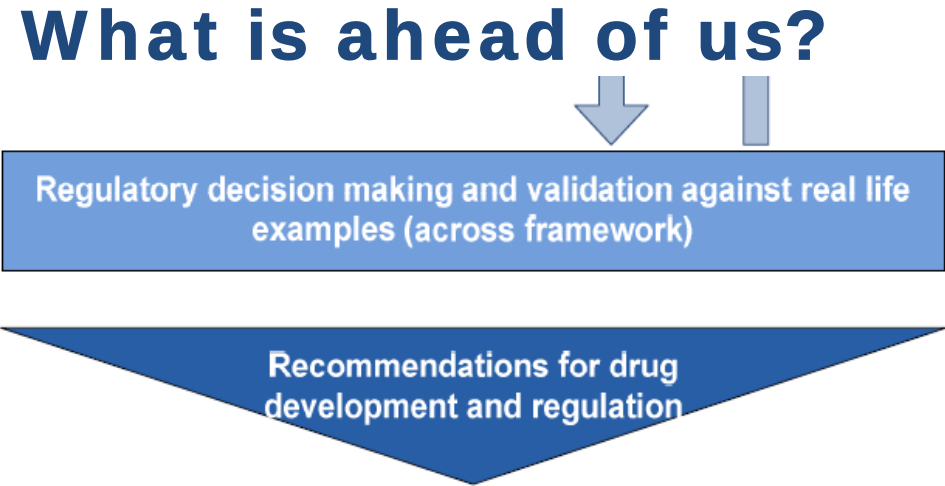


- Guidance on design at disease level not practical (over 8000 rare diseases).
- One general document may not provide sufficient guidance.
- Framework with intermediate approach, driven by key characteristics of medical conditions and treatment.
- Developed based on about 100 EMA dossiers.

Key output: Framework



What is ahead of us?



Regulatory decision making and validation against real life examples (across framework)

Recommendations for drug development and regulation

Ongoing research:

- In depth study of European Public Assessment Reports in orphan drugs.
- Support potential of guidance for existing methodology and potential application of new methodology.
- Using clustering of conditions as framework.

What is ahead of us?



Patient Think
Tank

- Generate information on key methodology for patients (patient leaflets).
- Patient perspective in recommendations.

Next Gen and
application

- About 8 PhD students to graduate.
- Many already involved in rare disease clinical trial projects (oncology, cystic fibrosis, ALS, transplantation medicine, Fanconi,....).

Towards guidance for practice



Asterix will finish October 2017, with important results still to follow.

Together with IDeAL and InSPiRe lay the ground work for renewed guidance.

