Possible scenarios for patients' panels, and open questions

A proposal by patients' advocates

ACT EU – Accelerating Clinical Trials in the EU

ACT EU Multi-stakeholder Platform AG meeting 17/09/2026

Goals:
With patients' panels,

•1

- Establishment of a high-quality dialogue between patients, sponsors and regulators

•2

- Patients inform sponsors on the design and the practical aspects of **innovative** trials

•3

- Patients and regulators align their views on scientific advice to be given
- (and HTA?)

•4

- Faster trial and significant amendments authorisation, recruitment and retention are facilitated

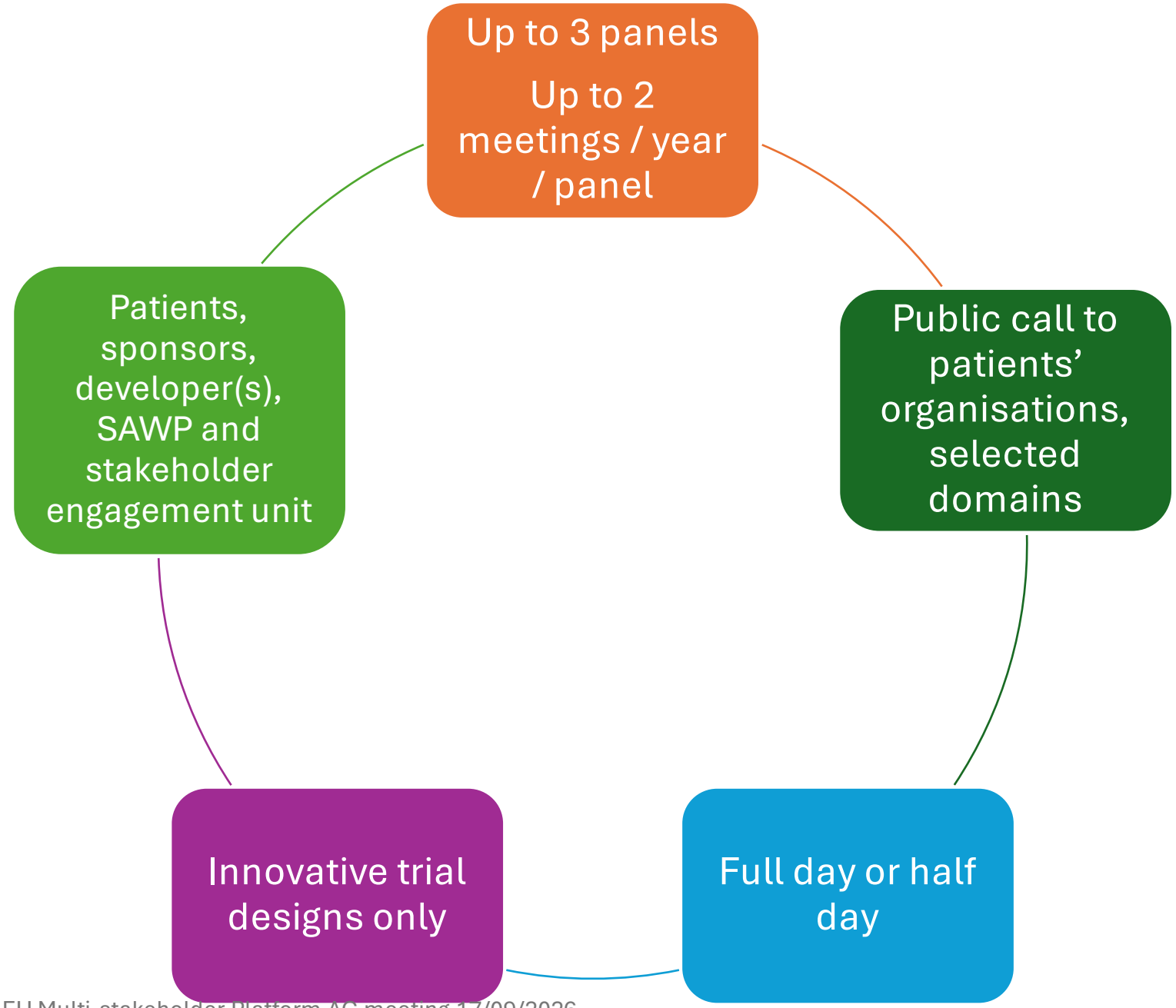
•5

- Trial participants are informed before, during and at the end of the trial

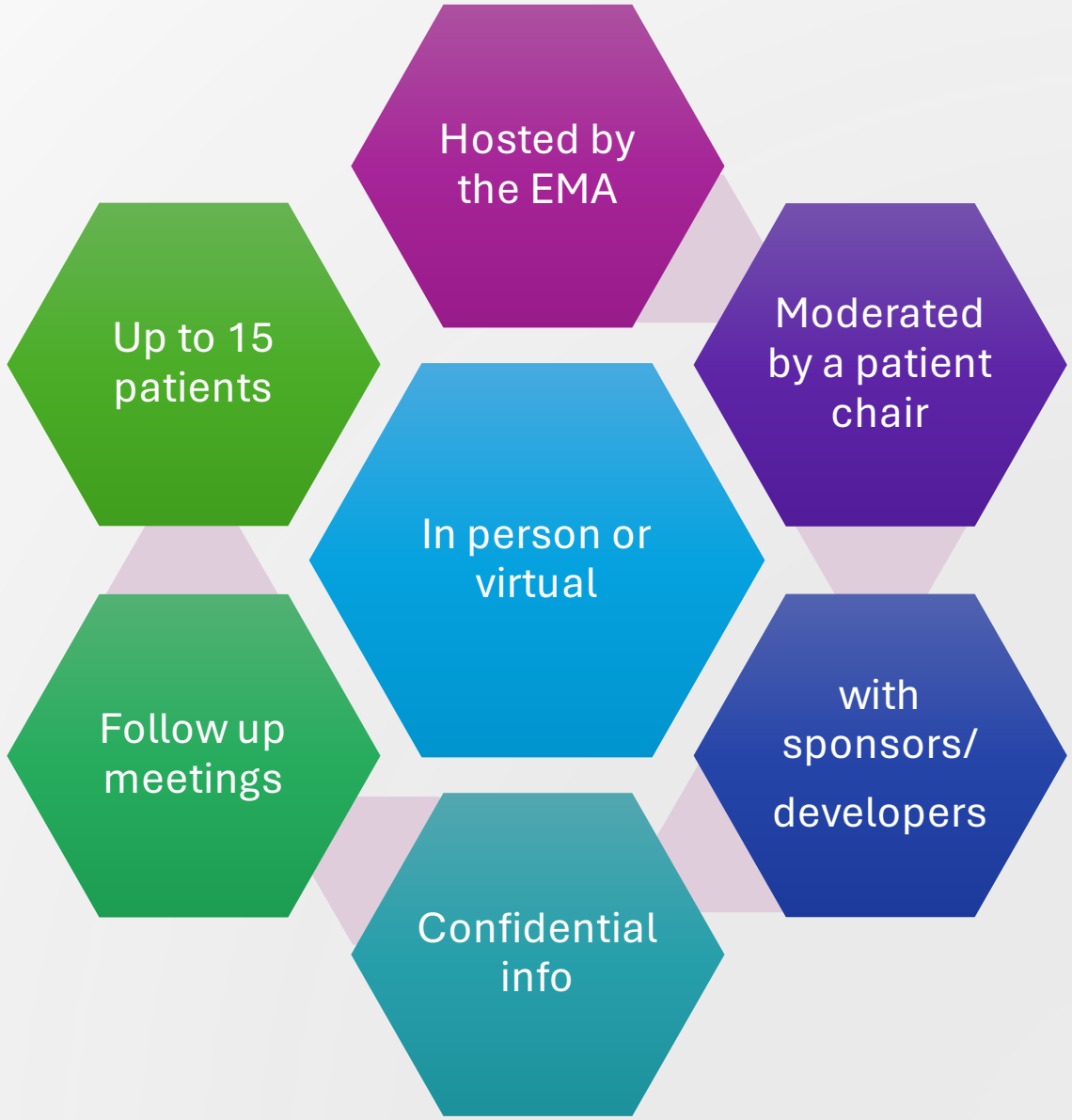
Why these panels?

Involvement of patients in industry or academic trials exist already	• But difficulties exist even when there is an excellent dialogue (eg Platform trials, where multi-lateral discussions are needed) • There are disease areas with no R&D in progress due to the complexity of designing trials. Time can be gained if all parties seat at same table
Patients' panels would complement existing forums where patients and sponsors interact on CTs (Community Advisory Boards, industry own Advisory Boards…)	• They would convene in specific situations (complex or highly innovative trial design), in some disease areas, where they add value, and when a larger group of patients' opinion is needed compared to SA (1-2)
With industry sponsors in particular: patient advocates engaged with sponsors in CT design / conduct are considered as consultants by EMA policy on managing competing Interests (Policy 044)	• This limits the possibility CAB members can engage with EMA, HTA, payers at EU or at National level • Not all patients' groups have the possibility to reserve some advocates • When organised and hosted by EMA, and regulators are present, these discussions fall under EMA activities exactly like other SA, SAG procedures

Hypothesis for the pilot



Practical arrangements



Which selected domains? High unmet needs?

Amyotrophic lateral sclerosis

- In the last 20 years: > 50 products tested, 2 authorised (but limited effect, or for 1%)

Pancreatic cancer

- Short life expectancy after diagnosis

Paediatric-onset rare diseases

Sanfilippo Syndrome, Phelan-McDermid Syndrome, Angelman Syndrome, Rett Syndrome…

Huntington disease

- Severe, complete dependency in daily life, which results in patients requiring full-time care, and finally death. The most common cause of death is pneumonia, followed by suicide.

Diseases with an existing CAB already, or to create a patients' panel from scratch (+6 months)?

- Multiple Sclerosis, as atypical haemolytic uremic syndrome, cystic fibrosis, Dravet syndrome, limb-girdle muscular dystrophy, asthma, bronchiectasis, chronic obstructive pulmonary disease , Psoriasis, Duchenne Muscular Dystrophy, Huntington, HIV…

Open domain among diseases benefiting from a PRIME product (selected 2S2026)

- And if a network of patients exists, or to create one from scratch?

Preparatory work

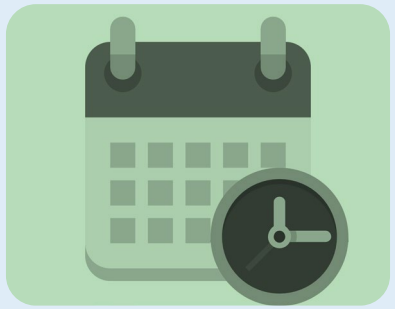
Patients' training

EUPATI

OpenAcademy (Summer School on R&D)

Confidentiality agreements

EMA template = same for all sponsors



Meeting agenda

Co-developed with sponsor

Meeting documents

Sent 2 weeks ahead

Other provisions

EMA rules for experts apply (DoI, allowance…)

Outstanding questions
A focus group might help

Organisation

- Can Patients' Panels be involved both in the design and in the conduct of trials ?
- Can Patients' Panels be involved in the explanation of the trial results to trial participants, and how?
- Could trial participants contact patient representatives in the Patients' Panel?
- Budgetary aspects for the pilot

Impact

- When do we know patient involvement is meaningful, or when it is tokenism? Impact measurement
- What do regulators expect to see reported in terms of patient involvement?
- Should methods be the same for private and for academic research ?

EU Patients' Panel in the global context

- Some protocols are global. Should engagement be regional, global? How to liaise Patients' Panel with other CABs?
- Communication between Patients' panel and Ethics Committees, Data Safety Monitoring Board / Independent Trial Review Committees

Next steps

STEP

To identify potential patients' networks (via a call, or based on needs from SAWP, committees or PRIME, or industry)

STEP

To evaluate how Patients' panel operate, discuss the pilot' outcomes, address challenges, adapt the pilots

To set an ACT EU focus group
To explore different solutions, to prepare the 1st pilot for 1Q2027

STEP

First pilot launched, 1st panels' meet 1Q2027

STEP

Based on outcomes, to develop a concept paper for the EMA management board for future activities

STEP

Your questions, reactions?