EnprEMA & ACT EU workshop on paediatric clinical trials

12 May 2026, 10:00- 17:00 (CET)

Hybrid meeting (EMA and online)

Welcome and introduction

Ricardo Fernandes *(EnprEMA co-chair and STAND4Kids)*

Gunter Egger *(EnprEMA co-chair and EMA)*

Peter Arlett *(EMA)*

12 May 2026

Session 1: Addressing key considerations

Moderators: Anette Solli Karlsen (NOMA) and Monique Al (CCMO and MedEthicsEU)

12 May 2026

Challenges for adolescent inclusion during the EU Clinical Trial Applications for a global Phase 3 study

Tim De Smedt

EU Regulatory Affairs Lead Neurology, UCB

12 May 2026

When does a pediatric phase 1 trial has a ‘therapeutic’ intent?

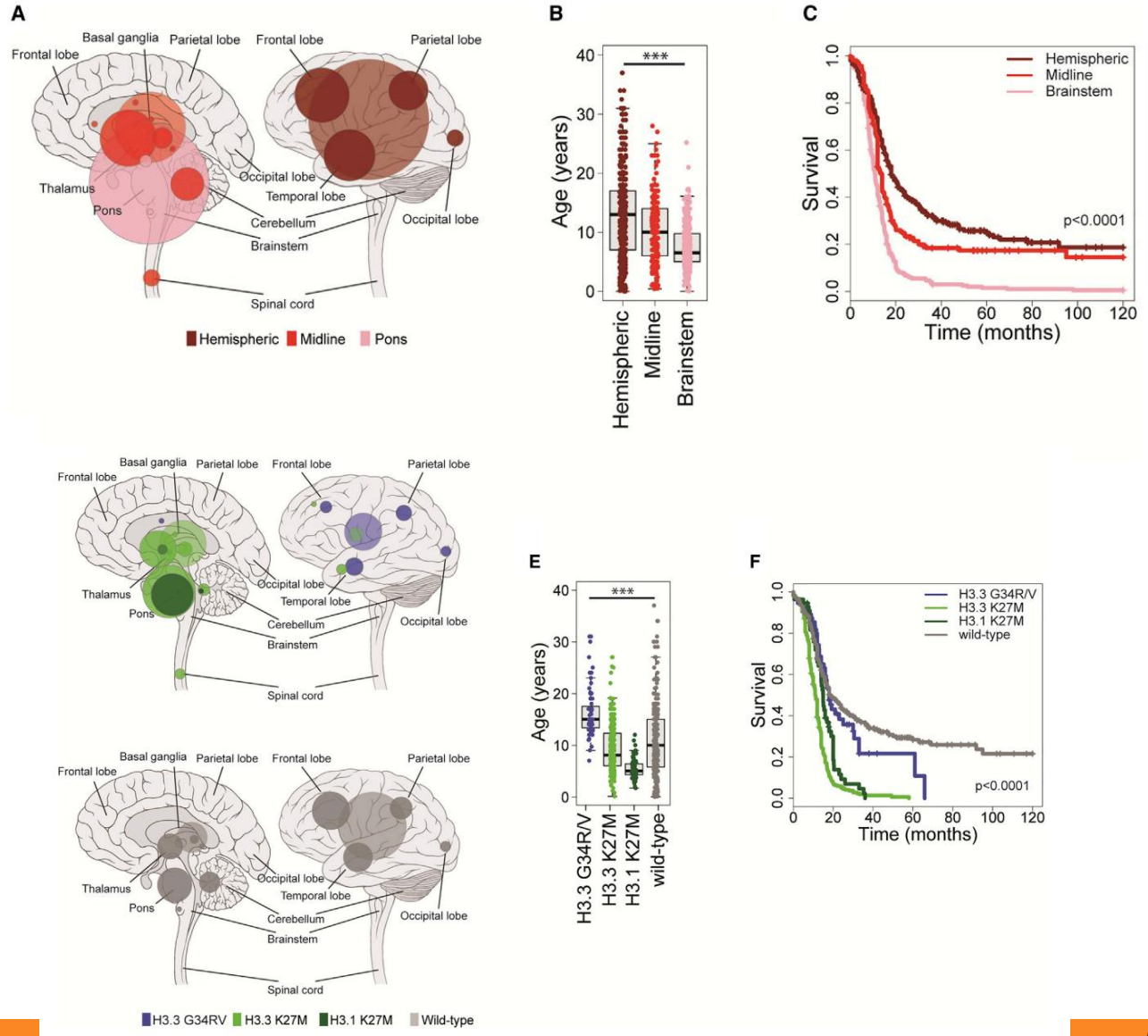
PNOC023: Transatlantic phase 1 trial with ONC206 for pediatric DMG patients

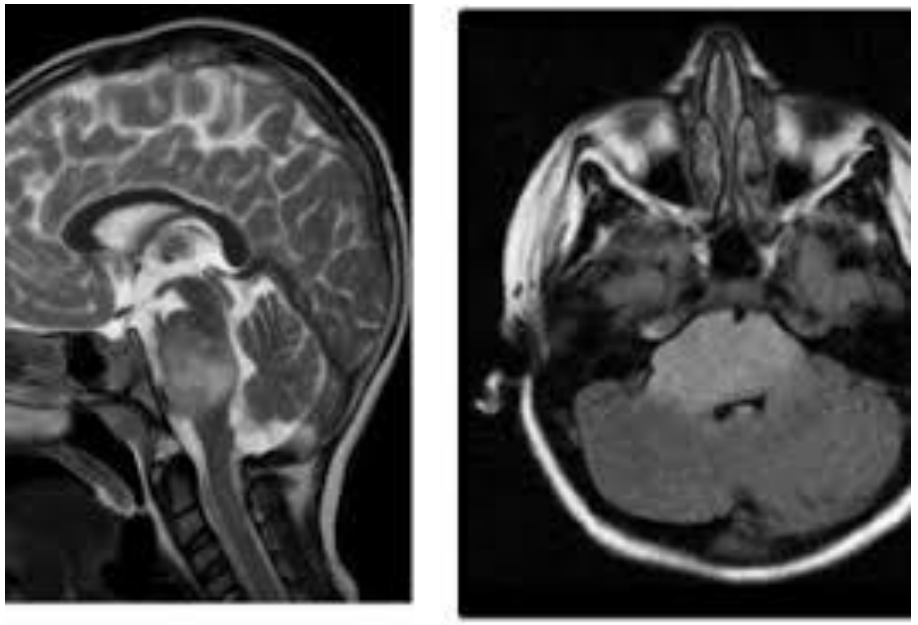
EnprEMA workshop

12th of May 2026

dr. J. van der Lugt and dr. T.C. Godschalk

The population





Nature Genetics paper van Filbin/Madge et al. (2022)

Background: ONC201 approved for DMG

PNOC022: A Combination Therapy Trial using an Adaptive Platform Design for Children and Young Adults with Diffuse Midline Gliomas (DMGs) including Diffuse Intrinsic Pontine Gliomas (DIPGs) at Initial Diagnosis, Post-Radiation Therapy and at Time of Progression

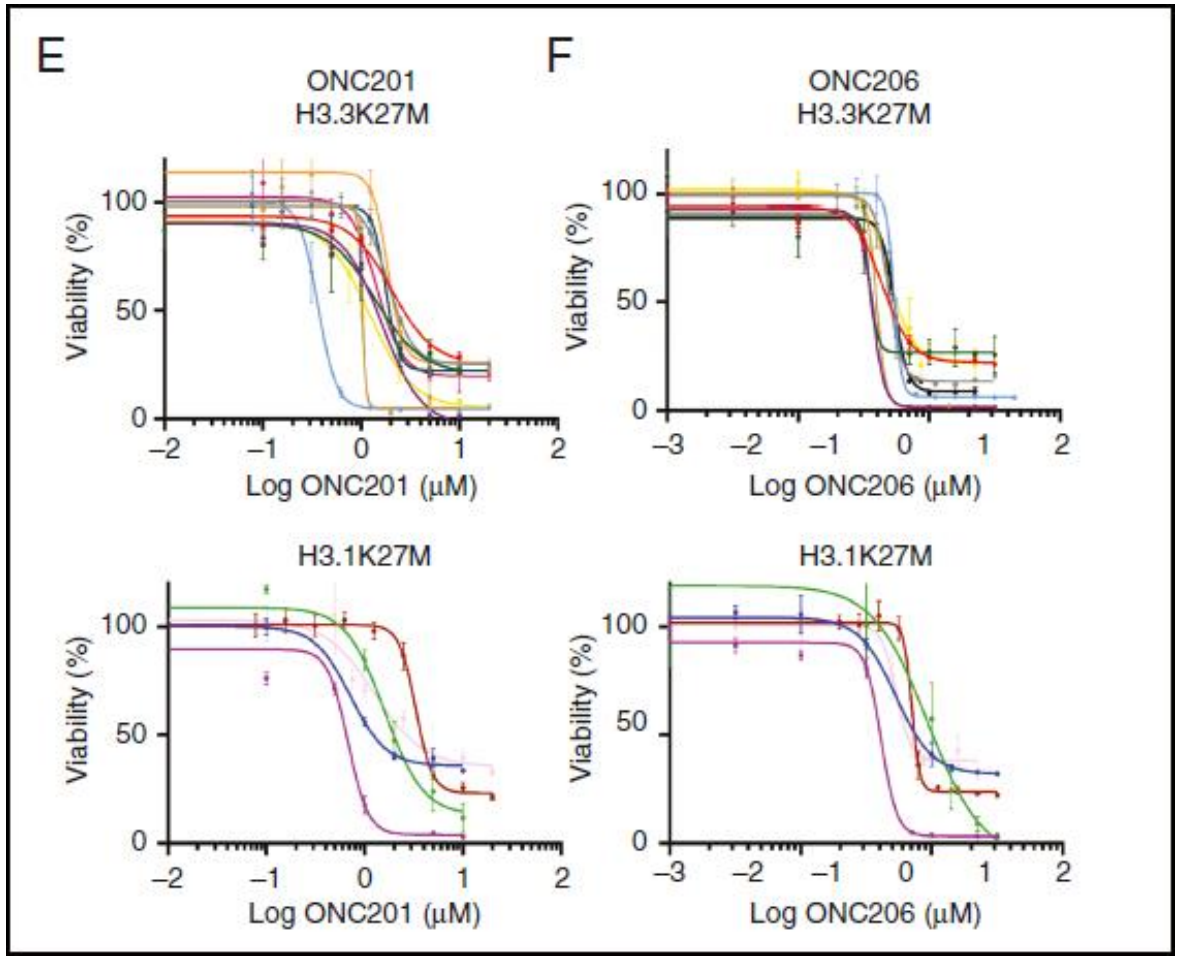
Included treatment with **ONC201**, phase II trial, was open at our site

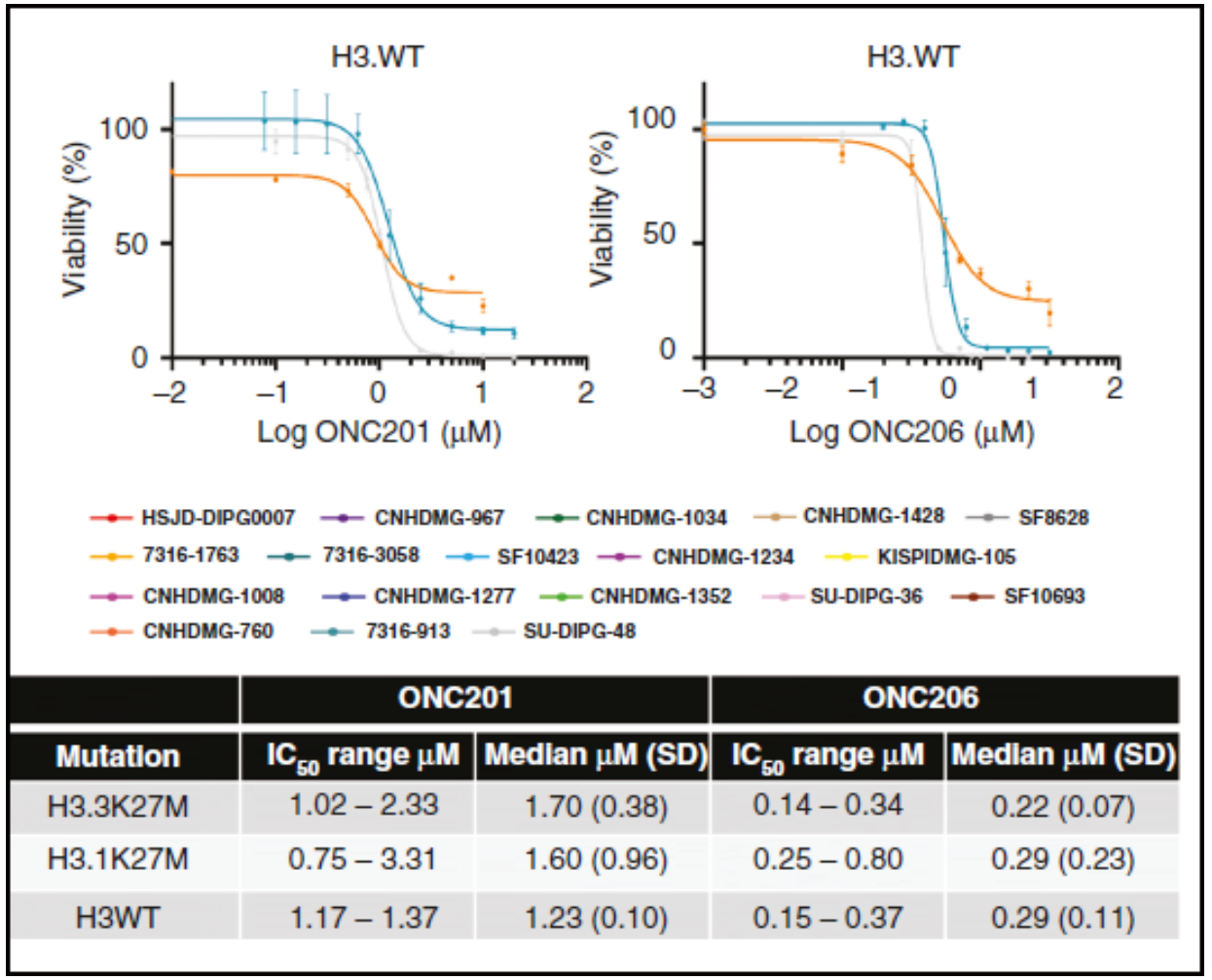
FDA grants accelerated approval to dordaviprone for diffuse midline glioma

On August 6, 2025, the Food and Drug Administration granted accelerated approval to dordaviprone (Modeyso, Jazz Pharmaceuticals, Inc.), a protease activator, for adult and pediatric patients 1 year of age and older with diffuse midline glioma harboring an H3 K27M mutation with progressive disease following prior therapy.

This represents the first FDA approval of a systemic therapy for H3 K27M-mutant diffuse midline glioma.

Background: ONC206 lower IC_{50} than ONC201 pre-clinically

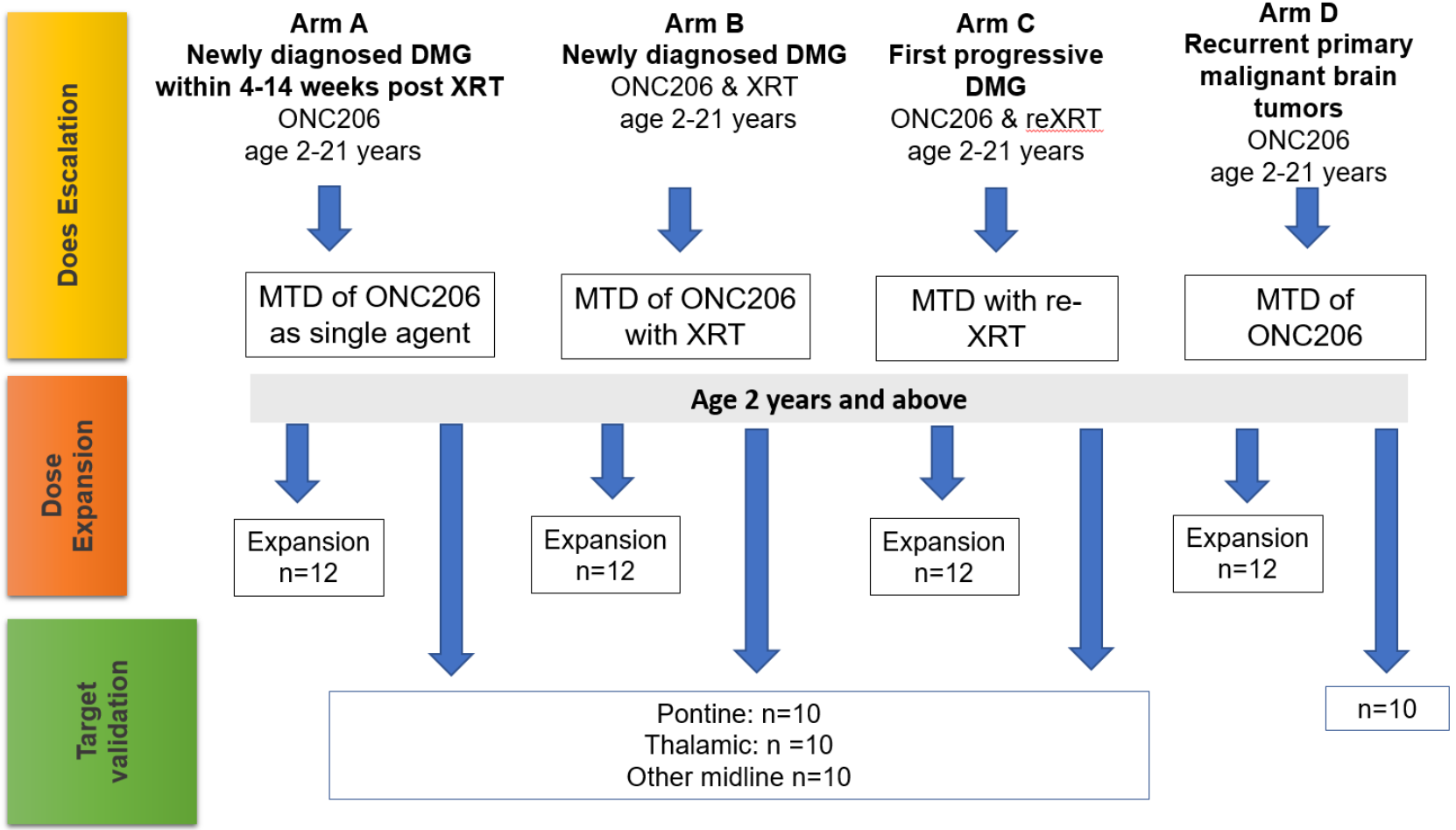




	ONC201		ONC206	
Mutation	IC_{50} range μM	Median μM (SD)	IC_{50} range μM	Median μM (SD)
H3.3K27M	1.02 – 2.33	1.70 (0.38)	0.14 – 0.34	0.22 (0.07)
H3.1K27M	0.75 – 3.31	1.60 (0.96)	0.25 – 0.80	0.29 (0.23)
H3WT	1.17 – 1.37	1.23 (0.10)	0.15 – 0.37	0.29 (0.11)

Background: ONC201 approved for DMG

PNOC023: Ope
Adults with Ne
Primary Maligr
EU CT: 2025-5



d Young
r Recurrent

Primary Objectives

Arms A & D:

1. To determine the safety and tolerability of ONC206
2. To determine the MTD of ONC206 as *single agent*

Arms B & C:

1. To determine the safety and tolerability of ONC206
2. To determine the MTD of ONC206 in combination with *radiation therapy*

Dose levels: At time of submission DL7 (no DLTs)

Table 8 Dose levels for participants based on body weight of 60 kg and above. Weight adjusted dosing is outlined in Appendix F.

Dose level	Dose of ONC206 in mg for participants 60 kg and above	Frequency	Dose mg/kg
0	25	Once weekly	0.42
1*	**50**	Once weekly	0.83
2	100	Once weekly	1.68
3	150	Once weekly	2.5
4	200	Once weekly	3.3
5	250	Once weekly	4.2
6	50	Twice a day Day 1, 2 and 3 of each week	0.83
7	150	Once a day, Day 1,2 and 3 of each week	2.5
8	100	Twice a day Day 1, 2 and 3 of each week	1.68
9	150	Twice a day Day 1, 2 and 3 of each week	2.5
10	200	Twice a day Day 1, 2 and 3 of each week	3.3

*Starting dose level

Final decision CA (the Netherlands) on PNOC023

The committee has decided that the **clinical trial is authorised with conditions:**

- To consider study as therapeutic and allow participation of children <12 years: **critical to evaluate preliminary efficacy results of the ongoing trial with ONC206**
- No preliminary data provided during the evaluation of submission + sponsor indicates that **preliminary efficacy of ONC206 is one of the exploratory objectives** of this trial: administration of ONC206 is considered a non-therapeutic intervention

→ **Therefore, the study is only approved for participants of ≥12 years old.**

RFI: assessment phase

RFI: **Please present the clinical results** (both safety and preliminary efficacy, e.g. decrease in mortality) **of phase I dose escalation with ONC206 in *adults* (NCT04541082) and discuss these results in context to the current outcomes for these different patient groups (in PNOC023)**.

Sponsor response:

There have been no DLT's to date on the PNOC023 trial. **Preliminary efficacy results are not available at this time while we remain in the dose escalation phase.**

(DSMC approvals per arm/dose level submitted not included in this ppt)

RFI: assessment phase

RFI: **Please provide additional information from clinical studies comparing the efficacy of *ONC201* in adults and children with preliminary efficacy data from the ongoing studies using *ONC206*.** Furthermore, please justify the additional benefit that is to be expected from ONC206 when compared to ONC201.

Sponsor response:

- As this is the first in children phase 1 trial with ONC206, data is **not available to provide at this time to compare the efficacy of ONC201 to ONC206 in humans.**
- The current trial still remains in *dose escalation phase* and is ongoing. ONC206 functions preclinically as “more potent” than ONC201 and therefore hypothesized to have increased antitumor activity→ reference to the paper

RFI: follow-up RFI (related to RFI's just presented)

RFI: **To consider the study as therapeutic and thereby allow children <12 years to participate, it is critical to evaluate preliminary efficacy results of the ongoing trial with ONC206:**

- Please provide the preliminary efficacy data of ONC206 for evaluation
- Discuss these results in context of the current outcomes for the proposed patient groups and compare these results with the efficacy results of ONC201.

Sponsor response:

- This study – in dose escalation phase - is to evaluate the MTD across Arms A-D and the phase 1 trial design is not built on efficacy. Page 46 of protocol: "There will be a *dose expansion phase* for Arms A-D to include at least 12 patients on the MTD per arm to further evaluate toxicity and preliminary efficacy"
- Efficacy endpoints are exploratory objectives on this trial. We hope to answer this question in the context of the ongoing study
- This study is not a comparable cohort to efficacy results of ONC201 and this data is not available. Preclinical data: ONC206 is at least as effective as ONC201 *in vivo* (mouse model) and clearly more potent *in vitro (*~10× lower IC_{50} values)

→ Therefore, one can argue that ONC206 is at least non-inferior and likely superior in intrinsic anti-tumor activity. ONC201 has shown some benefit clinically and based on these results has obtained FDA approval for relapse DMG. **Considering the same mechanism of action, it is anticipated that there may be therapeutic benefit for the patients even in the phase 1 setting. Therefore, we consider it as justified to include patient < 12 years of age.**

Points of discussion

- Why not beneficial for the patient?
- First in child possible?
- Fatal disease (of importance?)
- Discrepancy with other regulators (Swiss medic/FDA)?
- Mechanism of action argument?

Back-up slide:

ECTR article 32: Clinical trials on minors

A clinical trial on minors may be conducted only where, in addition to the conditions set out in Article 28, all of the following conditions are met:

(g) there are scientific grounds for expecting that participation in the clinical trial will produce:

(i) a direct benefit for the minor concerned outweighing the risks and burdens involved; or

(ii) some benefit for the population represented by the minor concerned and such a clinical trial will pose only minimal risk to, and will impose minimal burden on, the minor concerned in comparison with the standard treatment of the minor's condition.

Special patient populations at UCB

Generating evidence must be embedded early in drug development, rather than postponed until after approval

- Historically underserved patient groups in clinical research
- Strong commitment to balancing protection from research, vs. **protection through research**

Paediatrics

Women of Childbearing Age

Elderly

EU-level alignment did not prevent national concerns during the CTA

Clinical trial

- **Objective:** generate efficacy and safety data in adult + adolescent population (≥12 years), in outpatient setting
- **Design:** Phase 3 randomised, double-blind, placebo-controlled, study + open-label extension
- **Study drug:** caregiver- or patient-administered dosing in real-world context
- **Conduct:** >180 sites globally (EU, US, China, Japan,…)

Prior regulatory alignment

Recruitment of adolescents had already been **agreed with CHMP and PDCO**:

- CHMP scientific advice (completed prior to CTA)
- Paediatric Investigation Plan:
  - Day 60 PDCO report was available at the time of the Clinical Trial Application (CTA)
  - Final PDCO positive opinion became available during the CTA process

National challenges

Globally, the trial was approved as planned, including adolescents

In EU, 2 (out of 8) National Competent Authorities challenged adolescent inclusion, mainly because of:

- **National legislation** on:
  - inclusion of children in clinical trials
  - use of placebo in children
- **Risk management** in outpatient setting

Clarifications during the CTA resolved the challenge in 1/2 EU countries

Country 1: **Approved the study, with delays**

Legal basis of the challenge:

- A clinical trial in minors may only be performed **if absolutely necessary**
- Country 1 considered that **sufficient data could be generated in adults**, and adolescent participation was therefore not agreed

Key elements in the Company response during the Clinical Trial Application (CTA):

- Clear unmet medical need in adolescents, requiring data generation specifically in this population
- Prior CHMP and PDCO alignment
- Data supporting safety and efficacy for paediatric use of the study drug
- Safeguards in the study design, including for the outpatient setting

Country 1 eventually agreed that the potential benefits outweighed the risks

Clarifications during the CTA failed to resolved legal issues in EU Country 2

Country 2: **Did not approve inclusion of adolescents**

Legal basis of the challenge:

- Clinical trial may not include patients under 18 years of age if a comparable treatment exists in the indication
- Adolescents cannot receive placebo during an acute condition
- Efficacy and safety objectives must be demonstrated in adults first
- Discomfort with potential treatment decisions by caregivers in an outpatient setting

Key elements in the Company response during the CTA (multiple rounds):

- Same response strategy as for Country 1, with additional emphasis on:
  - Lack of suitable treatments and active comparators (ie, placebo use ≠ withholding necessary medical care)
  - Availability of well-established rescue treatment in case of acute events
  - Consistent approvals received globally (7 other EU countries, incl. Country 1, US, Japan, China)

Country 2 only approved an amended protocol, with recruitment limited to adults

- A follow-up discussion during study conduct, when additional data had accumulated, did not change the decision

Conclusions

Local regulatory frameworks

Local regulations can fragment paediatric evidence generation in EU:

- Can occur even for CHMP/PDCO-endorsed paediatric approaches and study designs

Placebo control

Use of placebo in paediatric studies remains a sensitive Legal and Ethical issue:

- Can occur even when use of placebo is scientifically justified and does not deny treatment

- CTA delays/refusals add feasibility challenges that can jeopardize paediatric inclusion, and ultimately, timely access to innovative medicines
- Multistakeholder initiatives and local harmonization could enhance EU competitiveness in clinical trials and facilitate early paediatric development

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

The choice of controls for medicinal products with invasive mode of administration – lessons learned (so far)

Understanding challenges in designing fit for purpose paediatric clinical trials

EnprEMA & ACT EU workshop on paediatric clinical trials

Presented by Maria Sheean on 12 May 2026
Scientific Officer at the Paediatric Medicines Office, Evidence Generation Department, EMA

An agency of the European Union

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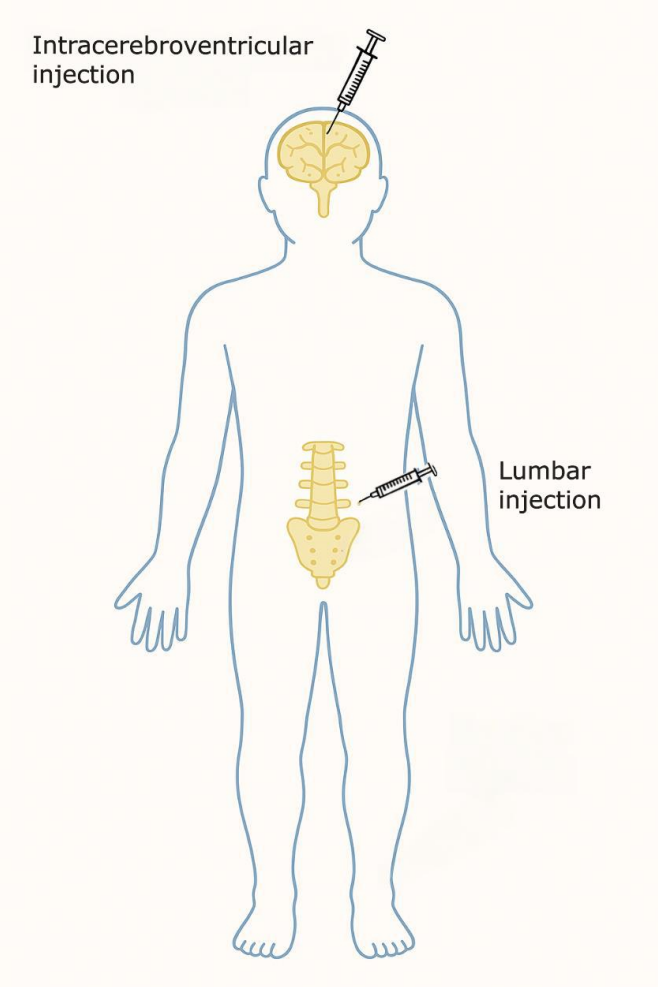
Problem statement



Paediatric Committee at EMA faced with PIP applications for **global developments** (regulatory alignment is challenging when the paradigm shifts)

Increasing number of products, either **AAV-mediated gene therapies** or **oligonucleotides** administered via IT or ICV injection targeting developmental conditions such as Dravet syndrome, Angelman syndrome and Rett syndrome.

Challenges: **vulnerable population,** rarity and heterogeneity of the disease, lack of predictive biomarkers, increasingly competitive research space

Definitions

ORIGINAL ARTICLE

Nusinersen versus Sham Control in Infantile-Onset Spinal Muscular Atrophy

Authors: Richard S. Finkel, M.D., Eugenio Mercuri, M.D., Ph.D., Basil T. Darras, M.D., Anne M. Connolly, M.D., Nancy L. Kuntz, M.D., Janbernd Kirschner, M.D., Claudia A. Chiriboga, M.D., M.P.H., +14 , for the ENDEAR Study Group* Author Info & Affiliations

A placebo control - intrathecal administration of an inert substance (e.g. artificial CSF) via a true lumbar puncture performed identically to the active treatment arm.

A sham-LP control - mimics all key procedural steps of a lumbar puncture without intentional dural puncture or intrathecal administration of the investigational product or fluid. Involves CSF sampling (low volume).

A prick-sham control - a superficial needle prick or skin puncture only, without advancement toward deeper spinal structures, dural penetration, or entry into the intrathecal space.

Learnings from Stakeholder interactions

Developer's perspective – PIPs for Dravet and Angelman syndrome

- **Placebo control, sham or delayed treatment sham-control** proposed for the main confirmatory trial
- Placebo mostly justified by the need for safety characterization, the need for biomarker research and the need for maintaining the blinding (DS and AS)
- Sham in more expensive and operationally challenging and thus less favoured by applicants

Learnings

In some cases, safety aspects already flagged in Phase 1 studies, development of biomarkers remains a hypothetical goal without concrete options

Patient community consultations

- Majority of respondents raised **concerns about placebo** within Angelman community BUT accepting the need for placebo-controlled trials not to risk 'product not reaching the market'.
- Dravet community seems to have more regulatory experience (more treatments available) and raises **safety concerns around LP** procedures
- Considerations around the **risks of sedation** are different, with Dravet community concerned about the risk of inducing status epilepticus

Learnings

Disease context matters! Extent of patient representative education/regulatory awareness makes an impact. Patient/carer education is needed

Paediatric clusters

- FDA and EMA processed submissions from applicants in parallel (often with FDA having a head start) and sometimes arriving at different wording of the guidance despite largely overlapping scientific discussions.
- Common commentaries were shared with applicants for two products, for which the applicants interpreted regulatory advice as conflicting.

Learnings

Prospective discussion between international regulators to foster harmonised global developments is needed (rare disease context!).

This applies also to European stakeholders involved in the application of the Art. 32 of the CTR.

PDCO/SAWP and parallel SAWP/HTA consultations

- PDCO/PME highlighted feasibility and ethical considerations in the context of a mainly scientific discussion at the SAWP
- This resulted in a more nuanced advice wording for several advices
- PDCO perspective valuable, raising HTA bodies awareness, as they will decide on the patients access to novel medicines.

Learnings

Early dialogue with the SAWP and HTAs is beneficial. Multistakeholder awareness training on the interpretation of the EU CTR is of value.

Key considerations for control choice

Key considerations when deciding on the control (placebo vs. LP-sham vs. skin prick-sham)

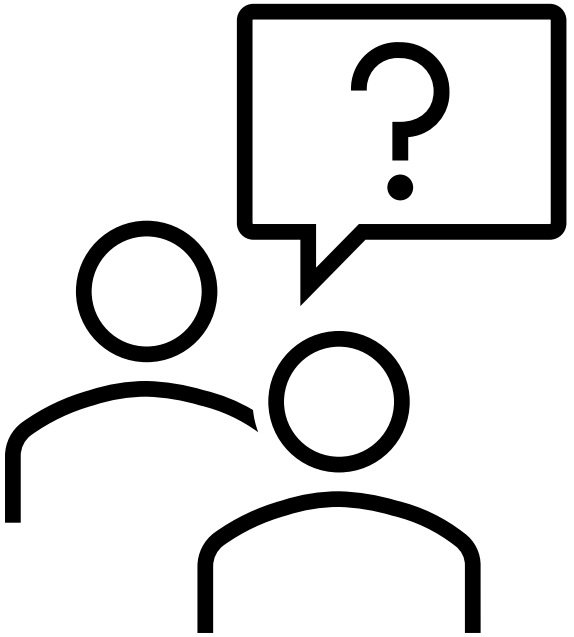
- What is the foreseen frequency and total number of procedures in the study?
- Is the control posing an unnecessary burden or risk to patients?
- Is CSF aspiration scientifically justified by planned safety characterisation, biomarker study? Is there a scientific benefit of a more invasive control?

Key considerations when deciding on the control (placebo vs. LP-sham vs. skin prick-sham) cd.

- What is the objectivity of the primary endpoint?
- What are the alternatives for controls?
- Is it technically possible to maintain study blinding?
- What is the planned/necessary duration of the trial?

Questions to the discussion panel

1. What additional tools would help in achieving global harmonization?
2. How to improve risk acceptance for diseases with no treatment, fast progression and huge unmet medical need?
3. To biobank or not to biobank CSF from trials? Can biobanking overcome ethical/risk challenges?



Any questions?

Further information

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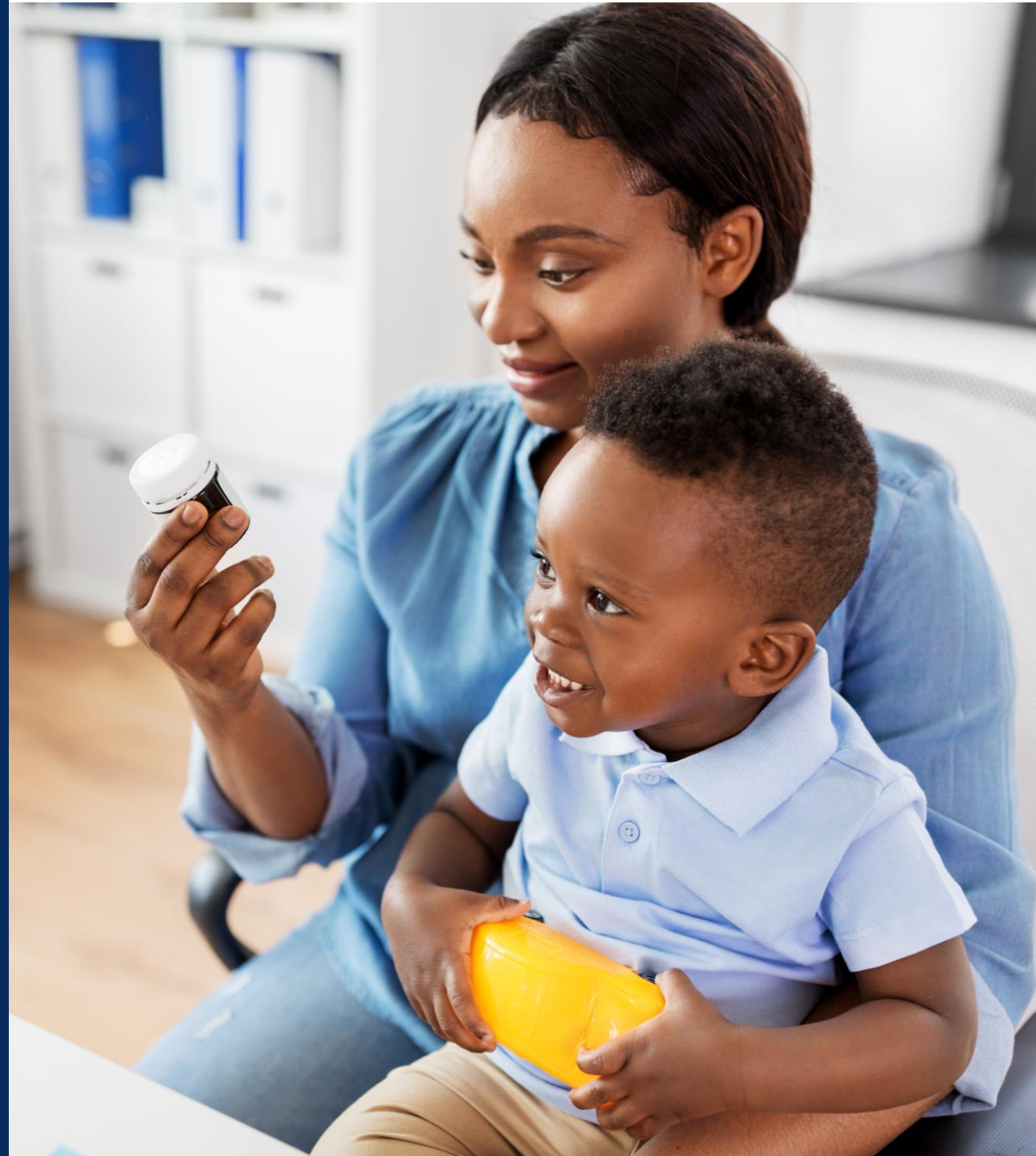
Panel discussion and Q&A session

Panellists
Industry: Angeliki Siapkara
Regulatory (NCA): Domenica Sorleti
Academic/network: Pirkko Lepola
Academic: Marek Migdal
Ethics: Michel Zwaan
Patient: Tomasz Grybek

12 May 2026

Feedback from breakout sessions + Q&A

Moderators: Ricardo Fernandes, Juan José Abellán



EnprEMA & ACT EU workshop, 12 May 2026
BOS 1: Paediatric platform trials and academia-industry collaboration (Rapp: Pamela Kearns)

- **Aim:**
  - to understand the prerequisites that facilitate the effective delivery of academic-industry collaborative platform trials for childhood diseases.
- **Background:**
  - Paediatric drug development often trails adult studies by many years, delaying access to urgently needed medicines.
  - Platform trials could accelerate progress, but operational, regulatory challenges and intellectual property concerns remain major barriers - prompting recent multi-stakeholder efforts to develop solutions.

C3PT Initiative: Collaborative Childhood Cancer Platform Trials Blueprint and Toolkit

A **framework for** implementation of **academic-industry collaborative studies** that **deliver data that could support a regulatory requirement (PIPs, iPSPs) and potentially marketing authorization applications.**

Defining the Purpose of the Platform

Decision tree to determine stage in drug development life cycle and the **value proposition the platform will deliver**

Regulatory Interaction

Co-development from concept to trial design with **regulators' endorsement** of platform design

Proactive selection of assets entering platform

Platform-associated **Asset Assessment/Evaluation Process**

PIP & iPSP

Guidance for **templated common sections** of regulatory documents

Trial Conduct Infrastructure

Operational Models and associated essential expertise and networks

Suggested shared and delegated responsibilities

Contracting Red lines

Standardised approaches to difficult topics: Data ownership, data sharing and usage, IP

Suggested principles and model clauses

Funding Models

Funding of platforms with **both non-commercial and commercial investments and what needs to be funded and by whom**

Expressing the Value Proposition

Guidance on

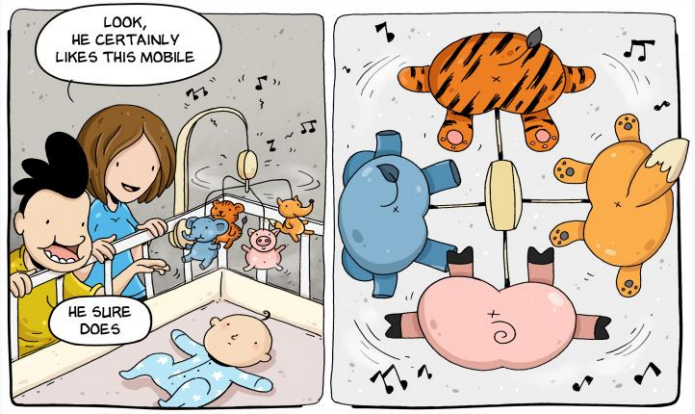
interacting with Industry

&

Marketing of the Platform to Pharma /Biotech

BOS 1: Discussion Points

- **Operational/Governance**
  - Contracting main challenge
    - Confidentiality
    - Financing (‘fit for filing’, national differences);
    - Securing commitment from Industry at the start when not clear how the data will be uses
- **Selection and prioritization of assets**
  - Competition between companies
    - Mitigated by Academic Sponsored trials as a neutral zone
  - Limited commercial interest
  - Lack of innovations for some diseases /conditions
- **Patient-centricity**
  - Need a meaningful range of perspectives from patients/advocates throughout
  - Resourcing can be a barrier
  - Cultural differences in willingness to have patient engagement
  - ‘arm allocation’
  - Communication is key



***Credit Vladimir Lopatin**, used with permission: https://www.instagram.com/piterskii_punk/*

BOS 1: Key messages

- What platform trials could unlock for paediatric development (speed/efficiency/equity)

- Critical success factors - governance, contract agreements, data sharing, sustainability (funding), stakeholder alignment, intrinsic patient advocacy throughout with diversity of perspectives

- How to adapt the oncology ‘Blueprint’ to other diseases (what transfers/what needs tailoring)

- Unmet needs/gaps identified
  - Lack of innovations for some diseases /conditions
  - Different prioritization/ drug selection models
  - Sustainability /funding of infrastructure

BOS 1: Paediatric platform trials and academia-industry collaboration

- **Recommendations & actions:**

  Setup, delivery and application of collaborative (academia-industry) patient-centered platform trials in paediatrics:

  - Inform adaptation/transfer of key elements of C3PT paediatric oncology blueprint for other childhood diseases
  - Define key operational infrastructure for efficient /accelerated trial conduct
  - Establish clear pathways for meaningful patient advocacy involvement
  - Further explore methodological applications in concrete paediatric platform studies supporting identification of R&D priorities able to address unmet medical needs

EnprEMA & ACT EU workshop, 12 May 2026

BOS 2: Accelerating patient recruitment and retention (Rapp: Marek Migdal, Ricardo Fernandes)

- **Aims:**
  - to horizon-scan opportunities to improve paediatric recruitment and retention
  - to identify workstreams that support stakeholders and strengthen recruitment, retention, and patient engagement
- **Background:**
  - Recruitment and retention to clinical trials are central to the success of medicines development but remain especially challenging for many paediatric trials and sites.
  - Several new approaches to recruitment and retention have been identified recently which have the potential to be adopted more widely.

BOS 2: Accelerating patient recruitment and retention

- **Challenges:**
  - Limited patient pool, regulatory & ethical requirements, cognitive, emotional, and social factors driving parental and child decision-making, family and system-level participation burden
- **Strategic recruitment planning:**
  - Protocol development, trial feasibility & site selection, recruitment communication planning
  - EnprEMA recommendations on **preparedness of paediatric CTs**
  - EnprEMA recommendations on **quality criteria for paediatric CT sites**

BOS 2: Accelerating patient recruitment and retention

- **Approaches to facilitate recruitment may:**
  - Focus on different stages of the trial lifecycle
  - Be implemented by sponsors, sites, networks, and/or other stakeholders
  - Have various degrees of patient and family involvement and engagement
  - Be applied as single or combined approaches, within or across studies

BOS 2: Accelerating patient recruitment and retention

- **Recommendations & actions:**
  - Cristina de Juan
    - simulation use during preparation of the protocol
    - effective way of protocol improvement
  - Franz Schaefer
    - enrollment prediction network (based on results of the survey)
    - success of enrollment is based on patient trust in the medical team
    - multifactoral environmental medical model to support recruitment
  - Marek Migdal:
    - Importance of decentralized clinical trials-benefit for the patient
    - Cross border trials

  - Open Discussion:
    - Involvement of patients as early as possible, prior to enrollment > better retention
    - Need for improvement on access for information on clinical trials in the EU
    - Trial representativeness
    - Culture of research in the clinical and patient communities (particularly for referrals)

EnprEMA & ACT EU workshop, 12 May 2026

BOS 3: Methodological approaches tailored to paediatric medicine development

(Rapp: Dominik Karres)

- **Aim:**
  - to explore the opportunities associated with existing quantitative methodologies through the lens of paediatric development and specific contexts of use.
- **Background:**
  - Innovative quantitative methods are increasingly important for generating robust evidence, particularly in rare-disease settings.

  - **Methodological guidance is evolving** ICH E11A (extrapolation), ICH M15 (MIDD), ICH E20 (adaptive designs), reflection papers on RWD in non-interventional studies, single arm trials concept papers on Bayesian methods, external controls

BOS 3: Methodological approaches tailored to paediatric medicine development

- **Key messages:**
  - **Requirement to develop** offers **opportunities** for **innovation**
  - **Extrapolation** from adult data informed by Bayesian and quantitative methods Designing trials addressing research questions…

    … while **minimising unnecessary studies** in children
  - **Innovative trial designs** (e.g., platform trials) enabling **development prioritisation** in areas of unmet need – opportunity to bridging the ‘exploratory – confirmatory’ evidence gap

BOS 3: Methodological approaches tailored to paediatric medicine development

Topic 1: Paediatric Extrapolation (safety and efficacy)

- Bayesian and quantitative methods to inform extrapolation from adult data, framed by ICH E11A – designing trials that address research questions while minimizing unnecessary studies in children
- Early extrapolation concept and plan to support discussion with regulators
- **Unclear for sponsors which information to present to regulators to support a proposed design using Bayesian methods for extrapolation -> guidance is helpful**
- Potential challenges with external controls due to more heterogeneous patient populations and standards of care. Randomisation is valuable to mitigate risks of introducing bias
- Choice of external information to extrapolate from deserves careful considerations – challenging to find the balance between quantity and relevance of external data
- Methodological level of detail at the time of developing a PIP?
- **HTA acceptance – ensuring information flow/ transparency across life cycle and decision making points (eg from PIP to CTA to MAA to HTA)**
- Extrapolation of safety?
- Considerations to assess a PIP include aspects such as pharmacology and safety, and it should also address the legal requirement of generating evidence to support decision making
- Biomarkers may be needed in addition to main adult endpoints/ how to address age specific differences in relation to relevance of clinical endpoint
- **Efficiency gains: disease similarity is part of the extrapolation concept and agnostic to medicines -> once established (accepted by PDCO), could it be acknowledged for future medicines in the same indication?**
- PRO information might be relevant to understand disease similarity

BOS 3: Methodological approaches tailored to paediatric medicine development

Topic 2: Development prioritisation

- Innovative trial designs – including platform trials – to bridge the ‘explore – confirm’ gap, enabling efficient evidence generation and informed R&D decision making in areas of unmet need
- Academic platform trial (industry buy-in e.g. in oncology) can be very efficient and may mitigate competition for patients but are dependent on funding
- Idea more straightforwardly applicable to medicines within the same class for a given indication, coupled with Bayesian methods
- Platform trials – less flexibility (e.g. in terms of timing) for individual companies, this should be taken into account in PIP process.
- Governance and operational aspects of such a platform trial would also pose challenges

BOS 3: Methodological approaches tailored to paediatric medicine development

- **Recommendations & actions:**
  - Dissemination of **educational/training** content developed by the network and partners (tOEG on **extrapolation,** ICH) (e.g., webinar)
  - **Acceptance of disease similarity** (adults vs paeds) could be made more accessible
  - Further explorations of methodological applications **in concrete paediatric platform studies** supporting identification of R&D priorities able to address unmet medical needs
  - Highlight **success stories (e.g. successful platform trials)** to inform and inspire sponsors

Summary of key outcomes and follow-up actions

Moderators: Ricardo Fernandes, Gunter Egger and Laura Pioppo

12 May 2026

Summary of key outcomes and follow-up actions

EnprEMA & ACT EU workshop on paediatric clinical trials (12/05/2026)

Accelerating paediatric trials and paediatric drug development

- Our destination
  - Better medicines (and other interventions) for children
- Our trajectory
  - As accelerated, straightforward and predictable as possible
- Reminder from physics
  - Acceleration is a vector quantity, defined by magnitude and direction
  - It depends on the net forces acting on a body and its mass
- Learnings
  - Exert tailored forces purposefully, proportionately and adaptively

NASA/Getty Images

Pediatric trial landscape

- Rich and dynamic ecosystem
  - Initiatives, stakeholders, infrastructures, tools
  - Opportunities and challenges
- Transformation is a reality
  - Innovation to address needs
  - Distinct archetypes and paradigm shifts in pediatric drug development
- Pediatric specificities
  - From design to conduct and reporting
  - Involvement and engagement of children and families

ACT EU

- Recent publication of the revised ACT EU workplan: [ACT EU workplan 2026-2027]
- In the workplan, reference to more **inclusive** clinical trials – revised ACT EU objectives
- Collaboration with IHI funded project **READI** to broadly join forces on more inclusive clinical trials

Objective 1

Strengthen EU competitiveness by accelerated clinical research timelines, increased efficiency and enhanced regulatory convergence, in line with the Biotech Act proposal amending the Clinical Trial Regulation and the Clinical Research Investment Plan by:

a. Strong leadership and coordination on clinical trial authorisation and execution.

b. Optimising ethical oversight and further

public trust in clinical trials remains a cornerstone of EU clinical research.

c. Supporting the conduct of large-scale multinational clinical trials with broader geographical scope.

d. Reducing administrative burden, especially for academic and SMEs sponsors and increasing coordinated national implementation.

e. Increasing awareness of the resources available to design, manage and

Objective 2

Strengthening clinical trials with appropriate **inclusion of under-represented populations** such as children and women (including pregnant and lactating women) to deliver decisional evidence.

This should include clinical trials that answer research questions for **unmet medical needs**, rare diseases as well as vaccines and therapeutics for public health crises and pandemics.

Objective 3

Heighten the impact of European clinical trials through **excellent and coordinated regulatory and scientific advice** to trial authorisation, to support marketing authorisation and access throughout the medicine lifecycle.

ACT EU

Patient involvement:

- Continue dialogue with key parties, e.g., ACT EU MSP Advisory Group, Enpr-EMA
- Exploring the possibility to have disease specific **patient pannels**, *format* under discussion
- Building **trust** with patients' community, planned communication campaign on clinical trials and opportunities that they offer and a communication campaign around clinical trials summary of results
- Monitor use of the **trial map** and further enhancement

On innovation:

- use of **AI** to support innovation on clinical trial design
- analysis of clinical trials with **decentralised elements** (based on CTIS data)
- planned workshop on **platform** trial end of the year

ACT EU

Competitiveness

KPI for measurement against target aiming at:

- 500 more multinational clinical trials in 5 years to increase attractiveness
- 66% of trials recruiting within 200 days of CTA submission, to be faster in providing access to treatment
- Importance of access and analysis of clinical trials data
- Activities to accelerate patient recruitment, incl. on **contractual agreement**
- Important also to align with **IHI funded Realise-D** on matters on recruitment

Regulatory activities complementary to ACT EU

- CTCG/PDCO interaction to harmonise
- CTR Collaborate
- CTCG, MedEthicsEU and CTAG

Current EnprEMA Initiatives

- Paediatric Site Quality Criteria
  - Finalising shared quality standards and publishing guidance for paediatric trial sites
- Cross-Border Trial Participation
  - Identifying barriers (e.g., language) and preparing recommendations to enable international patient inclusion
- Paediatric Research Nurse Workforce
  - Mapping EU paed. research nurse roles and needs; supporting creation of a European research nurse network
- PPI & YPAG Strategy
  - Mapping PPI capacity and developing a structured PPI engagement framework

Closing remarks

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Thank you