GAPP Study

Integrated Governance and Innovative Methodologies

Eleonora Di Campi, TEDDY Network

TEDDY General Assembly & Scientific Meeting

18 - 20 September, 2026, University of Bari Aldo Moro, Bari, Italy

TEDDY - European Network of Excellence for Paediatric Clinical Research
Via Niccolò Putignani 178 - 70122 - BARI - Italy
www.teddynetwork.net · info@teddynetwork.net

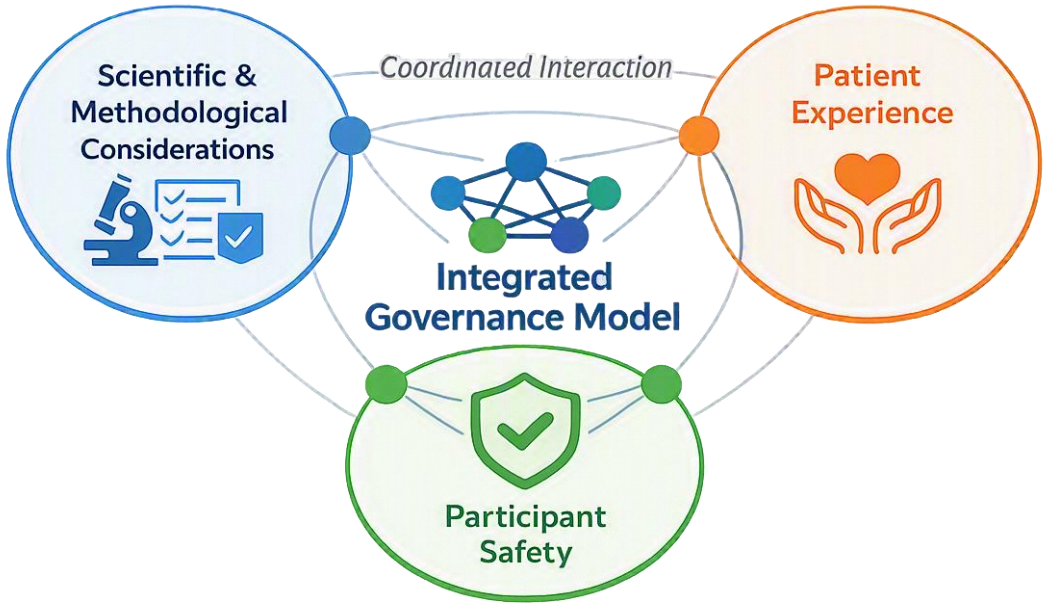
Scientific Background



The GAPP Study addresses a significant and longstanding challenge in paediatric clinical research:

The generation of robust clinical evidence in a heterogeneous and relatively small paediatric population affected by chronic neuropathic, nociplastic or mixed pain.

Scientific Background



To address this challenge, GAPP adopts an **innovative single-arm, Bayesian adaptive approach, combined with an individualized dose optimization strategy and an integrated governance model.**

This complexity requires that scientific and methodological considerations, participant safety and patient experience are not addressed independently, but are considered through **coordinated interaction** among the different governance bodies involved in the study.

GAPP: Governance Bodies

GAPP GAbapentin in Paediatric Pain

Within the GAPP Study, **3 Governance Bodies** have been appointed:



Data Safety Monitoring Board (DSMB)



Study Steering Committee (SSC)



Patient Advisory Board (PAB)

TEDDY NETWORK

GAPP: Governance Bodies: DSMB



The **Data Safety Monitoring Board (DSMB)** provides independent oversight of accumulating safety data and contributes to the protection of study participants throughout the conduct of the trial.

In an adaptive clinical study, **timely** identification and interpretation of emerging safety findings are particularly important, as safety considerations may influence decisions regarding dose optimisation, continuation of enrolment and other key aspects of study conduct.

GAPP: Governance Bodies: DSMB

GAPP GAbapentin in Paediatric Pain

DSMB currently includes 3 Independent Experts:

Data Safety Monitoring Board (DSMB)



Dr. Paola Baiardi
Biostatiscian
ICS Maugeri



Prof. Evelyne Jacqz-Aigrain
Prof. of Pharmacology – Emeritus
University Paris Cite - France



Dr. Andrea Michele Wolfler
Head of the Complex Operational Unit for Anaesthesia and Acute and Procedural Pain Management at the Gaslini Institute

TEDDY NETWORK

GAPP: Governance Bodies: SSC



The **Study Steering Committee (SSC)** provides scientific and strategic guidance throughout the study and supports the interpretation of emerging evidence in the context of the study objectives, methodology and clinical relevance.

Its role is particularly important in ensuring that the implementation of the adaptive design remains scientifically coherent and that operational challenges are appropriately addressed.

GAPP: Governance Bodies: SSC

GAPP GAbapentin in Paediatric Pain

Study Steering Committee (SSC)

The **Study Steering Committee (SSC)** includes the PIs of each involved clinical center, UCL and Sponsor representatives:



UCL

Prof. Oscar Della Pasqua

Chair of Clinical Pharmacology & Therapeutics at the University College London

TEDDY NETWORK

GAPP: Governance Bodies: SSC

The **Study Steering Committee (SSC)** includes the PIs of each involved clinical center, UCL and Sponsor representatives:





Dr. Anna Szumowska

Specialising in anaesthesiology and intensive care at Children's Memorial Health Institute, Warsaw.

INSTYTUT
„POMNIK-CENTRUM ZDROWIA DZIECKA"

GAPP: Governance Bodies: SSC

The **Study Steering Committee (SSC)** includes the PIs of each involved clinical center, UCL and Sponsor representatives:





Dr. Tom De Leeuw

Specialising in Paediatric chronic pain at Erasmus MC Sophia Children's Hospital

Erasmus MC
Sophia Children's Hospital

GAPP: Governance Bodies: SSC

The **Study Steering Committee (SSC)** includes the PIs of each involved clinical center, UCL and Sponsor representatives:

Study Steering Committee (SSC)



Dr. Justine Couturier-avez

Specialising in Paediatrics and Neuropaediatrics at Hospital Center University of Lille

CHU LILLE

GAPP: Governance Bodies: SSC

The **Study Steering Committee (SSC)** includes the PIs of each involved clinical center, UCL and Sponsor representatives:

Study Steering Committee (SSC)



Dr. Maria Giuseppina Petruzzelli

Specialising in Child Neuropsychiatry at University Aldo Moro of Bari.

UNIVERSITÀ DEGLI STUDI DI BARI ALDO MORO

GAPP: Governance Bodies: SSC

The **Study Steering Committee (SSC)** includes the PIs of each involved clinical center, UCL and Sponsor representatives:





Dr. Elmira Kola

Professor and pediatric intensive care specialist at Mother Teresa University Hospital Center, Tirana.

QENDRA SPITALORE UNIVERSITARE
"NËNË TEREZA"

GAPP: Governance Bodies: PAB



The **Patient Advisory Board (PAB)** provides the perspective of children, adolescents, advocates and caregivers and contributes to ensuring that the study remains understandable, feasible and acceptable for participants and families.

Its contribution is particularly relevant for:

- **evaluating participant burden,**
- **communication strategies,**
- **study procedures**
- **practical barriers to participation.**

GAPP: Governance Bodies: PAB



1. Muminah Aaisha-Seddika -**TEDDY KIDS Network - Moorfields eye-YPAG, United Kingdom**
2. Eirini Daskalopoulou -**TEDDY KIDS Network - KIDS Rare Hellas, Greece**
3. Francesco Meloni - **Italian Association of Bladder Exstrophy and Epispadias ODV**
4. Nepheli Mimi - **TEDDY KIDS NETWORK**
5. Martina Leandro - **TEDDY KIDS NETWORK - KIDS Bari**
6. Thuraya Layla Pearl Derby- **TEDDY KIDS Network - Moorfields eye-YPAG, United Kingdom**
7. Valeria Gatti - **TEDDY KIDS NETWORK - KIDS Bari**
8. Valentina Jalby - **TEDDY KIDS NETWORK**
9. Eleonora Passeri – **TEDDY KKIDS Network – KIDS Umbria**
10. Alexander Wiśniewski - **TEDDY KIDS Network - POLPEDNET KIDS**
11. Steisi Prenga - **TEDDY KIDS Network – KIDS Albania**
12. Martina Leonardo – **TEDDY KIDS Network – KIDS Bari**
13. Gabriella Monacelli – **TEDDY KIDS Network – KIDS Bari**
14. Clara Sim-Sim – **TEDDY KIDS Network – Ando Portugal**
15. Maria Codesido Garcia – **ADEE España**

Purpose of the meeting



The aim of today's session is to create a structured **multidisciplinary discussion** in which the DSMB, SSC and PAB can explore the main **opportunities** and **challenges** associated with the GAPP Study and its innovative methodological approach.

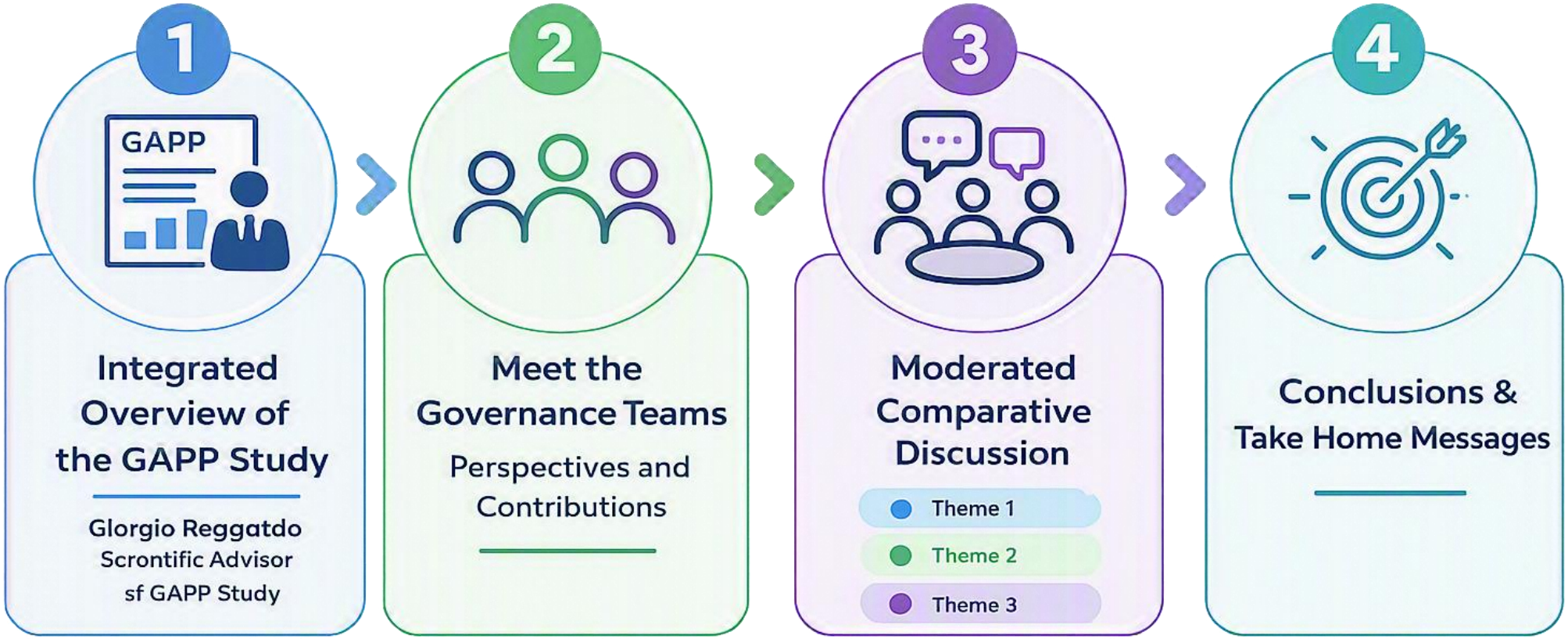
Purpose of the meeting

The discussion will focus on how the three governance bodies can contribute, from their respective perspectives, to:

- ensuring **participant safety** throughout the study;
- maintaining **scientific and methodological robustness**;
- ensuring that **study procedures remain feasible and acceptable for children, adolescents and their families**;
- identifying **potential challenges** before key study milestones and adaptive decision points;
- **strengthening communication and coordination** across the different governance structures.

GAPP Round Table: Structure of the Session

GAPP GAbapentin in Paediatric Pain



TEDDY NETWORK

GAPP Round Table



Moderated Comparative Discussion



Theme 1
Generating Robust Evidence in a Heterogeneous Paediatric Population

Theme 2
Adaptive Methodology, Safety and Decision-Making

Theme 3
Patient Experience and Feasibility

Moderated Comparative Discussion



Opening question: GAPP brings together children and adolescents with different ages, clinical profiles, pain characteristics, comorbidities and treatment histories.

How can we ensure that this complexity is appropriately reflected not only in patient inclusion, but also in the generation and interpretation of evidence?

Moderated Comparative Discussion



Questions for the SSC

- Which sources of heterogeneity are most likely to influence treatment response, adherence or the interpretability of the study results?
- How should comorbidities and concomitant treatments be considered when interpreting treatment response and study outcomes?
- Are there particular methodological considerations that could help ensure that the study population remains clinically meaningful and representative despite its heterogeneity?

Moderated Comparative Discussion



Question for the DSMB

- Are there aspects of clinical heterogeneity that may also have important safety implications and therefore require particular attention during the study?

Moderated Comparative Discussion



Question for the PAB

- From a patient perspective, what can help maximise the opportunity for children and families with different clinical needs and circumstances to participate in the study?
- Do age, pain characteristics, previous treatment experience or comorbidities influence how children and families experience participation?

Moderated Comparative Discussion



Opening question: GAPP uses a single-arm Bayesian adaptive design, allowing the study to learn from accumulating data and adapt predefined aspects of the study when appropriate.

How can this flexibility be combined with robust scientific oversight, careful safety assessment and transparent decision-making?

Moderated Comparative Discussion



Questions for the SSC

- What are the key methodological considerations when interpreting emerging data in a single-arm adaptive study, particularly in the absence of a concurrent control group?
- Which emerging findings or decision points should require particular scientific scrutiny, and how can scientific continuity be maintained if an adaptation is considered necessary?

Moderated Comparative Discussion



Questions for the DSMB

- In a single-arm study without a control group, how should emerging safety signals be interpreted, and what additional contextual information may be needed to distinguish expected events from signals requiring further investigation or action?
- What types of safety findings would require particularly close monitoring or potentially trigger an ad hoc review or action?
- Are there specific adverse events or safety findings that should be systematically reviewed during study implementation to minimise the risk of relevant events being overlooked or under-reported?

Moderated Comparative Discussion



Cross-committee question

- When emerging data raise scientific, methodological and safety considerations at the same time, how should the DSMB and SSC interact to ensure timely communication and coherent decision-making while preserving their respective roles and independence?

Moderated Comparative Discussion



Questions for PAB

- If the study evolves as evidence accumulates, how can patients and families be kept informed and meaningfully involved, rather than simply informed about decisions that have already been made?
- What would transparent and understandable communication look like when study results are reassuring, but also when unexpected findings or changes occur?

Moderated Comparative Discussion



Opening question: Scientific robustness is essential, but so is the ability of children and families to participate in the study without unnecessary burden.

How can we ensure that study procedures remain both scientifically necessary and as manageable and supportive as possible for participants?

Moderated Comparative Discussion



Questions for the PAB

- Which aspects of study participation could be improved to make the experience more comfortable, accessible and manageable for children and families?
- How important is the frequency of study assessments in determining the overall burden of participation, and how could this burden be minimised while maintaining the scientific and safety requirements of the study?
- Beyond the study procedures themselves, how can the relationship between healthcare professionals, children and families contribute to making participation feel more supportive, respectful and human?

Moderated Comparative Discussion



Questions for the PAB

- In particular, what could help make procedures such as blood sampling less burdensome and better understood by children and families?
- How can PROMs, PREMs and quality-of-life measures help us understand whether the study is making a meaningful difference from the perspective of children and families?

Moderated Comparative Discussion



Questions for the SSC

- How can patient feedback be translated into practical improvements during study implementation while preserving methodological consistency across participating sites?
- Are there aspects of participant feedback that should be considered particularly important when making decisions about study procedures or implementation?

Moderated Comparative Discussion



Questions for the DSMB

- Can participant burden become a safety, ethical or study-conduct issue, and how should this be recognised and considered alongside clinical safety signals?

Question to all participants:

- Looking ahead to study implementation, what is the one issue that each governance body believes should receive particular attention during the first phase of the GAPP Study?


thank
you!