Draft Agenda
Venue: Faculty Club; Leuven, Belgium

Thursday

13:00–14:00 Arrival and networking lunch (Room Van Hamaele)
Sandwiches, soup and coffee/tea

14:00–14:30 Opening plenary (Room Sint Barbara)
Introduction to the workshop objectives, overview of the current challenges in outcome measurement in DEEs, and presentation of the workshop structure. Participants and stakeholder groups will be introduced, followed by an explanation of the consensus methodology, breakout group structure, and the expected outputs of the meeting, including the development of a strategic roadmap and a consensus publication.

14:30–15:45 Session 1 – DEE trial landscape, natural history and implications for outcome measurement (Room Sint Barbara)
The session will begin with a brief introduction to Developmental and Epileptic Encephalopathies (DEEs), providing clinical context on disease heterogeneity, developmental trajectories, and the key challenges faced by affected children and families. This will be followed by an overview of the evolving clinical trial landscape, including traditional anti-seizure medication trials and emerging disease-modifying or gene-targeted interventions. Presentations will also summarize key insights from natural history studies and highlight how variability in developmental trajectories and disease progression complicates outcome assessment. Attention will be given to the implications of these factors for the selection, interpretation, and limitations of clinical outcome assessments (COAs) currently used in clinical practice and research. The session will conclude with a moderated discussion focusing on how trial design and natural history data should inform the development of meaningful and developmentally appropriate outcome measures.

15:45–16:15 Coffee break and networking (Room Van Hamaele)
Coffee, Tea and some sweets

16:15–17:00 Outcome Neuropsychology survey (EpiCARE) results (plenary) (Room Sint Barbara)
Presentation of the results of the EpiCARE Outcome Neuropsychology survey, highlighting cross-country variation in assessment practices across European centers, frequently used instruments, and areas of uncertainty or inconsistency in current clinical and research settings. The session will also provide a structured overview of the main categories of clinical outcome assessments (COAs) currently used or proposed in DEE research, including cognitive and neuropsychological testing, adaptive behavior scales, patient- and caregiver-reported outcomes (PROs), goal-based outcome measures, and participation- or functioning-based assessments. By combining survey findings with a methodological overview of available assessment approaches, this session will map the current outcome measurement landscape in DEEs and identify key gaps that will be explored in the subsequent psychometric and breakout sessions.

17:00–18:00 Psychometric foundations in DEE (plenary) (Room Sint Barbara)
This session will introduce key psychometric principles relevant to outcome measurement in children with severe neurodevelopmental impairment. Topics will include validity, reliability, and responsiveness of instruments in low-functioning populations; the impact of floor effects and restricted measurement ranges; differences between proxy-reported and performance-based assessments; and the interpretation of change, including the distinction between statistical and clinically meaningful change. Attention will be given to the challenges of measuring developmental trajectories and modeling longitudinal change in small

and heterogeneous DEE populations. The session will provide the methodological foundation for the subsequent breakout discussions on measurement barriers and interpretation of outcomes.

18:00–19:15 Breakout Session 1 – Measurement limitations in severe DEEs
This first breakout session will focus on identifying the main methodological limitations of current outcome measures used in children with severe developmental impairment. Participants will discuss practical and psychometric barriers that arise when applying existing instruments in DEE populations, with the aim of identifying priority areas for improvement in outcome measurement.

Group A – Floor effects and measurement range (Room: Anna De Paepe)
This group will examine the limitations of existing instruments in capturing functioning at the lowest developmental levels frequently observed in DEEs. Discussion will focus on the domains in which currently used measures lose sensitivity, the lowest reliably measurable levels of functioning, and whether adaptations such as alternative scoring approaches or modified administration procedures could extend the usable measurement range. The group will also explore which developmental domains are most vulnerable to under- or mis-measurement in severe DEE populations.

Group B – Responsiveness and interpretation of change (Room: Sint Gommarius)
This group will address how change can be detected and interpreted in children with severe developmental impairment. Participants will discuss how minimal clinically important differences might be defined in DEEs, how true individual-level change can be distinguished from measurement noise or developmental variability, and how developmental and seizure-related changes should be interpreted together when evaluating treatment effects.

Group C – Feasibility and assessment burden (Room Florquin)
This group will focus on the practical feasibility of outcome measurement in DEE studies. Discussion will address the acceptable level of assessment burden for children and families, the impact of fatigue and behavioral tolerance on the validity of assessments, and logistical challenges such as assessment duration, travel requirements, and access to specialized centers. Participants will also explore the feasibility of implementing standardized assessment protocols across different European healthcare systems.

20:00 Dinner (Room: Heilige Geesttafel)
4-course dinner

Friday

08:30–09:45 Session 2 – Trial design and endpoint strategy (plenary) (Room: Sint Barbara)
Overview of clinical trial designs in DEEs, including traditional anti-seizure medication trials and emerging disease-modifying approaches. The session will examine endpoint selection in different trial contexts (including basket and N-of-1 designs), regulatory expectations, statistical constraints in rare populations, and implications for the use and interpretation of developmental outcome measures. Moderated discussion.

09:45–10:15 Coffee break (Room Van Hamaele)
Coffee, Tea and some sweets

10:15–11:45 Session 3 – Patient and parent perspectives (plenary) (Room: Sint Barbara)
Two patient representatives will present structured perspectives on meaningful change, daily life priorities, and experiences with clinical trials, including perceived mismatches between trial endpoints and lived reality. An expert presentation will summarize current scientific evidence regarding priority domains of meaningful change in DEEs, including developmental

level, communication, language, psychomotor development, and transition to greater independence. Moderated discussion.

11:45–12:30 Networking Lunch (Heilige Geesttafel)
Lunch-buffet with quiches, salads and sandwiches
Waters, coffee/tea

12:30–14:00 Breakout Session 2 – Priority domains and unmet measurement needs
Building on the methodological limitations identified during the first breakout session, this session will focus on defining which developmental and functional domains should be prioritized in outcome measurement for DEEs. Discussions will address both the domains that should form the core of future outcome frameworks and the domains that are currently underrepresented or insufficiently captured in clinical trials and research.

Group A – Core developmental and functional domains (Room: Anna De Paepe)
This group will focus on identifying the developmental and behavioral domains that should be prioritized in outcome assessment for DEEs. Participants will discuss which domains are essential for capturing developmental and functional change in this population, how these domains should be prioritized across different levels of neurodevelopmental impairment, and whether certain domains may be considered broadly relevant across DEE syndromes. The group will also explore how a minimal set of core domains could be defined while allowing space for syndrome-specific or context-dependent measures.

Group B – Underrepresented domains and cross-cultural variability (Room: Sint Gommarius)
This group will explore developmental and functional domains that are currently insufficiently measured or overlooked in existing outcome frameworks for DEEs. Participants will discuss which aspects of functioning may remain underrepresented in clinical trials and observational studies, and how assessment practices vary across European centers and healthcare systems. Attention will be given to how contextual factors—such as access to rehabilitation services, differences in clinical infrastructure, or cultural expectations—may influence which outcomes are measured and how they are interpreted. The discussion will also consider which aspects of outcome assessment could realistically be harmonized across countries while remaining sensitive to differences in clinical practice and healthcare organization.

Group C – Meaningful change and alignment with family priorities (Room Florquin)
This group will examine how outcome measures can better capture meaningful change in the daily lives of children with DEEs and their families. Participants will discuss where current clinical trial endpoints may fail to reflect lived experience and which outcomes families consider most important but remain difficult to measure in existing frameworks. The group will also explore how developmental endpoints might better capture functional change across different stages of impairment and how definitions of meaningful improvement may vary depending on developmental level, family priorities, and care context.

14:00–14:45 Session 4 – Innovation and emerging technologies (plenary) (Room: Sint Barbara)
Overview of emerging approaches for outcome measurement in DEEs, including digital assessments, wearable technologies, passive data collection, eye-tracking, home-video analysis, and AI-supported analysis. The session will also explore hybrid and remote assessment models and their potential to reduce assessment burden while maintaining methodological rigor.

14:45–15:15 Coffee break and networking (Room Van Hamaele)
Coffee, Tea and some sweets

15:15–16:45 Breakout Session 3 – Methodological solutions and future directions
Building on the priority domains and measurement needs identified during the previous sessions, this breakout session will focus on potential methodological solutions for improving outcome measurement in DEEs. Discussions will explore how emerging technologies, methodological innovations, and analytical approaches could address current measurement limitations while remaining feasible and interpretable within clinical trials.

Group A – Standardization and adaptation of existing outcome measures (Room: Anna De Paepe)
This group will focus on how currently used developmental and neuropsychological instruments can be applied more consistently across studies and centers in DEE research. Participants will discuss challenges related to the use of existing test materials in children with severe neurodevelopmental impairment, including floor effects, administration difficulties, and interpretation of scores. The discussion will explore whether adaptations to administration procedures, scoring approaches, or interpretation frameworks could improve their applicability in DEE populations. The group will also consider how greater standardization across centers could be achieved while maintaining flexibility for different clinical contexts.

Group B - Digital and remote outcome measurement approaches (Room: Sint Gommarius)
This group will explore the potential role of digital tools and remote assessment strategies in improving outcome measurement in DEEs. Participants will discuss technologies such as wea rable sensors, eye-tracking, passive data collection, or structured home-video analysis, and consider their feasibility, validation requirements, and potential integration into clinical trial designs.

Group C – Validation and analytical approaches for complex developmental outcomes (Room Florquin)
This group will focus on the methodological and statistical frameworks required to ensure that outcome measures used in DEE research are robust, interpretable, and acceptable in clinical trial contexts. Participants will discuss the level of validation required for both existing and novel measurement approaches, strategies for modeling developmental trajectories in small and heterogeneous populations, and methods for handling missing data and participant drop-out in longitudinal studies. The discussion will also consider how developmental and seizure-related outcomes might be analyzed together while preserving interpretability for clinicians, researchers, and regulators.

17:00–18:00 Steering committee and rapporteurs meeting (Room: Anna De Paepe)
Synthesis of breakout outputs, identification of converging priorities, drafting of structured synthesis slides, and preparation of thematic pillars for the Saturday consensus and roadmap sessions. The steering committee will synthesize breakout outputs and organize them into thematic clusters that will serve as the basis for the structured consensus discussion on Saturday.

19:00 Networking Dinner (Room Heilige Geesttafel)
Reception-style diner with 9 little courses per person, drinks included

Saturday

09:00–10:30 Structured thematic synthesis (plenary) (Room: Sint Barbara)
Each breakout group will present a concise synthesis of their discussions (8 minutes per group), followed by moderated discussion. Presentations will focus on key findings from the breakout sessions, including identifiedmethodological limitations, priority domains for outcome measurement, and potential methodological solutions. Particular attention will be given to areas of convergence, remaining uncertainties, and actionable recommendations that can inform the development of a shared framework for outcome measurement in DEEs.

10:30–11:00 Coffee break and networking (Room Van Hamaele)
Coffee, Tea and some sweets

11:00–12:00 Roadmap construction (interactive plenary) (Room: Sint Barbara)
Building on the synthesis session, participants will collaboratively develop a structured roadmap for advancing outcome measurement in DEEs. The chairs will facilitate a structured consensus discussion in which key themes identified during the breakout sessions are progressively refined, grouped, and prioritized. Through moderated discussion and iterative prioritization, participants will identify short-term (1–2 years), medium-term (3–5 years), and long-term priorities.
The roadmap may address several key areas, including:

- harmonization and adaptation of existing outcome measures across centers
- development of standardized domain frameworks for DEEs
- methodological validation and regulatory readiness of outcome measures
- integration of innovative and digital assessment approaches

This session will aim to produce a clear set of strategic priorities to guide future collaborative research and clinical trial design.

12:00–12:45 Establishment of thematic working groups (Room: Sint Barbara)
Based on the roadmap priorities, participants will establish a small number of thematic working groups to continue collaboration after the workshop. Each working group will define its scope, identify a lead and co-leads, and outline initial milestones and follow-up activities.
Possible thematic clusters may include:

- harmonization and adaptation of existing outcome measures
- digital and remote outcome assessment approaches
- methodological validation and statistical frameworks
- cross-center collaboration and data harmonization

12:45–13:00 Closing (Room: Sint Barbara)
Final confirmation of workshop outputs, including the structure of the consensus publication, authorship principles, and timeline for manuscript development and follow-up activities.

Farewell lunch (Room Van Haemele)
Sandwiches, coffee, tea