



Global Full-service CRO

STUDY INSIGHTS

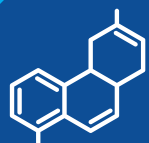
Multicentre Paediatric Acceptability Study for **Cyclophosphamide** Powder for Oral Solution (30 mg/mL)





Study Objective

To evaluate the overall acceptability, taste and smell acceptability, and oral-mucosal safety of Cyclophosphamide Powder for Oral Solution (30 mg/mL) in paediatric cancer patients receiving oral cyclophosphamide therapy.



Molecule Overview

Cyclophosphamide is a widely used alkylating antineoplastic agent indicated for multiple paediatric and adult malignancies.

Mechanism of Action:

Alkylating metabolites interfere with DNA replication, resulting in cytostatic and cytotoxic effects.

Clinical Need:

Conventional tablet crushing or dissolution may lead to dosing inaccuracies, poor palatability, reduced compliance, and an increased risk of medication errors.

Innovative Aspect:

A flavoured liquid formulation was developed to improve palatability, dosing precision, and patient compliance in paediatric populations.



Scope of work delivered by Lambda

A leading India based pharmaceutical company engaged Lambda Therapeutic Research Limited to conduct a paediatric acceptability study to support regulatory submission to the European Medicines Agency (EMA).

The study addressed an important unmet clinical need: the lack of an age appropriate oral dosage form of cyclophosphamide for children who experience difficulty swallowing tablets or require precise BSA based dosing.

The programme was designed in alignment with ICH E6 (R3) guidelines and paediatric ethical considerations while generating data to support formulation acceptability and safety.

Services Provided by Lambda:

- End to end clinical trial management
- Multicentre site selection, initiation, and monitoring across India
- Regulatory and GCP compliant study conduct aligned with EMA expectations
- Clinical data management and statistical support
- Maintenance of an audit ready Trial Master File (TMF)
- Clinical Study Report (CSR) preparation support



Study Overview

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| Study Design | <ul style="list-style-type: none"> • Prospective, multicentre, open label, non randomized acceptability study |
| Population | <ul style="list-style-type: none"> • Paediatric oncology patients aged 3 to <18 years • 20 subjects enrolled, including a minimum of 14 subjects aged 3 to 11 years |
| Study Duration | <ul style="list-style-type: none"> • Approximately 17 days per subject |
| Endpoints | <ul style="list-style-type: none"> • Primary: Overall formulation acceptance • Secondary: Taste and smell assessment • Safety: Oral mucosal tolerability |
| Assessments | <ul style="list-style-type: none"> • 5 point facial hedonic scale for formulation acceptance • WHO oral mucositis grading |
| Regulatory Standards | <ul style="list-style-type: none"> • EMA submission support • Conducted in alignment with ICH E6 (R3), Declaration of Helsinki, and paediatric ethics guidance |



Challenges & Solutions

| Challenges | Solutions Implemented |
|---|--|
| <p>Slow recruitment in a paediatric oncology population:</p> <p>Recruitment was challenging due to the limited availability of eligible paediatric oncology patients.</p> | <p>Lambda strengthened investigator engagement and implemented structured parent/LAR counselling to clearly explain study procedures and objectives.</p> <p>Flexible screening timelines and proactive site follow up helped maximize enrolment opportunities across participating centres.</p> |
| <p>Daily IMP Administration Visits (10-Day Continuous Visit Requirement):</p> <p>The requirement for continuous daily visits created logistical and compliance challenges for patients, caregivers, and study sites.</p> | <p>Lambda optimized site workflows to reduce overall visit duration and coordinated study visits with routine clinical care wherever feasible.</p> <p>Continuous caregiver engagement and regular communication supported subject adherence and retention throughout the study period.</p> |
| <p>Difficulties in Smell and Taste Assessment</p> | <p>To improve consistency, age appropriate visual analogue scales (VAS) with simplified descriptors were used during assessments.</p> <p>Caregiver/LAR support was incorporated to facilitate accurate responses, with greater emphasis placed on overall palatability trends rather than isolated sensory observations.</p> |



Key Findings

- Primary and secondary endpoints were successfully assessed across all dosing days.
- The liquid formulation demonstrated improved patient acceptance compared to tablet dispersion
- No unexpected safety signals related to oral mucosa or tolerability were observed
- The study was conducted with high protocol compliance and strong data integrity
- Data generated were considered suitable to support EMA regulatory submission
- Audit ready documentation and complete safety reporting were achieved



Conclusion

Lambda Therapeutic Research successfully delivered this paediatric acceptability study for Cyclophosphamide Powder for Oral Solution, supporting the development of an age appropriate formulation for paediatric oncology patients.

Through integrated clinical operations, paediatric focused study execution, and regulatory aligned processes, Lambda generated robust acceptability and safety data to support global regulatory submission.

Connect with us to explore how we can accelerate your clinical development programs.

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