



Research & Innovation
Clinical Research Centre



HRB HRB Clinical Research Centre
Health
Research
Board

CHI Clinical Research Centre

An exciting time for the future of child health and paediatric research in Ireland

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Clinical Trials Start Up Manager

Overview

- Change
- New ecosystem for paediatric research
- The CHI-CRC
- Case Study : Neuromuscular paediatric clinical trials

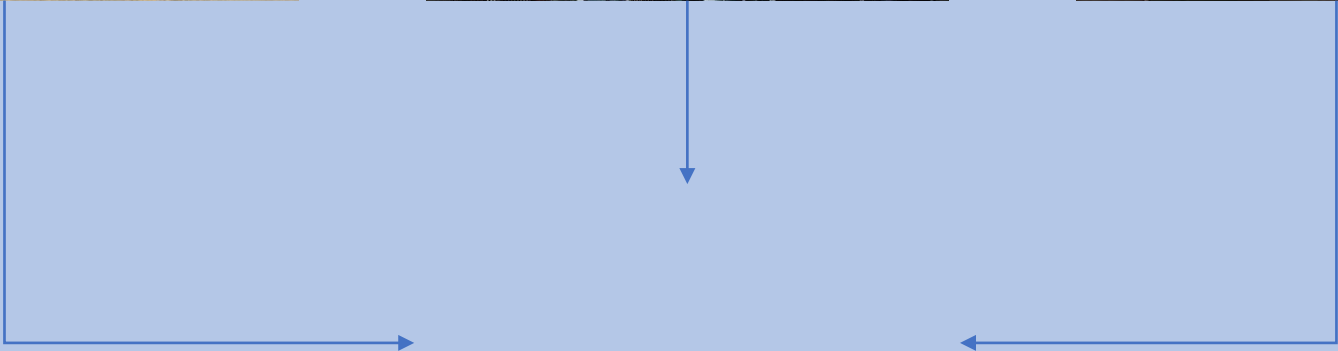


It's All Change

New Model of Care for Paediatrics in ROI	✓
Establishment of Children's Health Ireland	✓
New academic environment in paediatrics	✓
New Research & Innovation Department	✓
New Research Governance & Management Structures	✓
New CHI-Clinical Research Centre	✓
New Facilities	



Original locations: Children's Health Ireland



Children's Health Ireland



In 2023

CHI at Tallaght



CHI at Connolly



CHI at Crumlin



Sláinte Leanaí Éireann

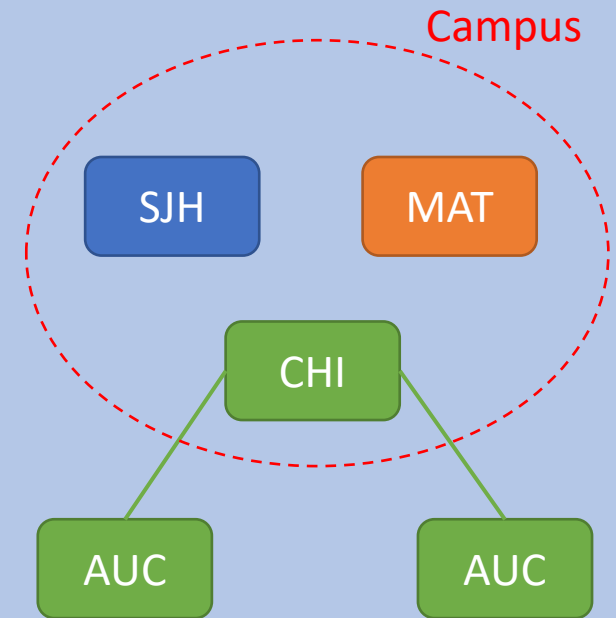


Children's Health Ireland

CHI at Temple Street



Moving closer towards...



New ecosystem for paediatric research

- Unique context, no single academic partner
- Focus on integration of clinical care, research and education
- New structures for Research Governance, Management and Support
 - PAHSC
 - Research & Innovation Office
 - CHI-CRC
 - CRIC
- New systems and processes
- Expanded staff





The CHI-Clinical Research Centre



- Launched in 2022, thanks to co-investment from HRB and CHF
- Vision: To become a leading International Centre for Clinical Trials in Children
- Cross-site research centre – staff work as one team across CHI sites
- Expanded capacity and new capability

VISION

To become a leading International Centre
for Clinical Trials in Children

AIMS

- High Levels of Activity and Access
- All Trials in CHI Integrated
- Advanced Skill Sets in Place
- Sustainable Structures in Place

OBJECTIVES

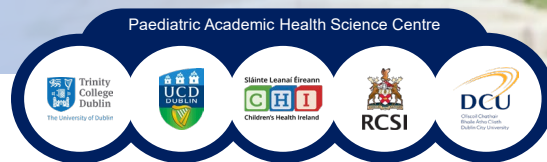
- Communicate
- Train
- Develop
- Sustain



Facilities

National tertiary children's hospital

- New hospital 470 bed - single rooms, NICU, PICU, 93 day beds
- **Research Facilities**
- Dedicated Clinical Research Facility at new hospital
- +Integration of research with clinical care - Research space at ED, ICU etc.
- CRIC
- Shared campus with SJH: partnership model for CRF/Cs
- CHI at Connolly – access to facilities at new RCSI Clinical Research Centre



New Facilities

Lecture Theatre

Clinical Research Facility
(Upper Floor)



Development of the new Clinical Research Facility





Services & Supports



Biostatistics & Trial Methodology



Study Startup & New Business Development



Research Nursing



Quality Management System



Funding & Grant Applications



Project Management & Study Coordination



Trial Pharmacy



Research Training



Public & Patient Involvement



Regulatory Affairs & Ethics



Data Management

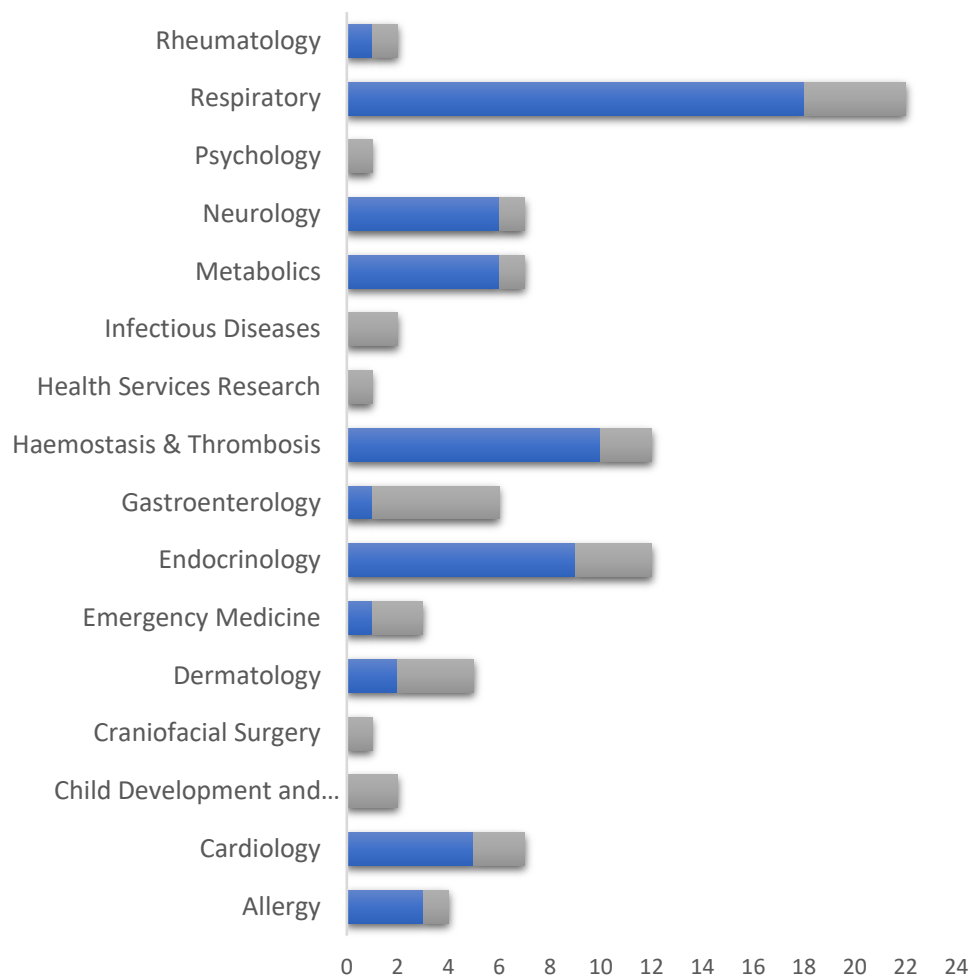


Research Lab

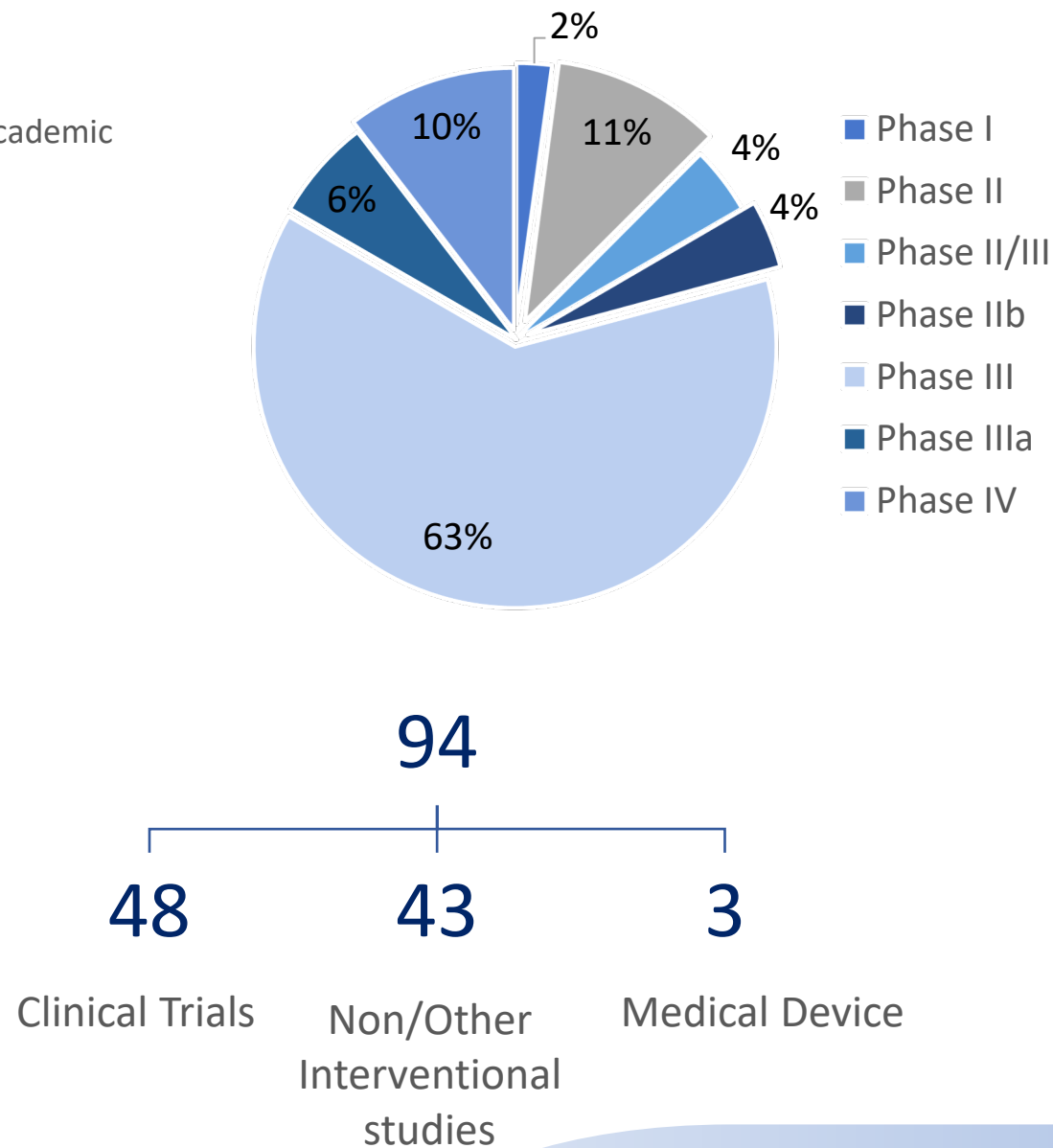


CHI CRC Activity

CHI CRC Activity by Disease Area ■ Industry ■ Investigator-led/Academic



CHI CRC CTIMP Activity by Phase



Case Study: Neuromuscular Clinical Trials

- CHI at Temple Street is the Centre for Neuromuscular clinical trials in Ireland
- Neuromuscular Clinical Trials Research Team working with Dr Declan O'Rourke, Consultant Paediatric Neurologist (Associate Director of CHI CRC)
- Duchenne Muscular Dystrophy, is a rare genetic neuromuscular disorder causing progressive muscle loss, primarily in young boys. The disease is characterised by alterations in the protein dystrophin, which is crucial for building muscle connections
- 5 CTIMPs in DMD since 2017 and more in the pipeline



Experienced Neuromuscular Clinical Trials Team

Phase II/III portfolio

- A Phase III Double-blind, Randomized, Placebo-Controlled Study assessing the Efficacy, Safety and Tolerability of Idebenone in Patients with Duchenne Muscular Dystrophy Receiving Glucocorticoid steroids (SIDEROS).
- A Randomized, Double-blind, Placebo-Controlled, Global Phase 3 study of Edasalonexent in Paediatric patients with Duchenne Muscular Dystrophy.
- An Open-Label Extension Study of Edasalonexent in Paediatric Patients with Duchenne Muscular Dystrophy.
- A Double-Blind, Placebo-Controlled, Multicenter study With an Open-Label Extension to Evaluate the Efficacy and Safety of SRP-4045 and SRP-4053 in Patients With Duchenne Muscular Dystrophy.
- A Randomized, Double-Blind, Dose Comparison Study Evaluating the Safety and Efficacy of Two Dose Levels of Eteplirsen
- A Phase 2 Randomized, Double-blind, Placebo-controlled, MAD study with Open-label Long-term Extension in male participants with DMD amenable to exon 51 skipping





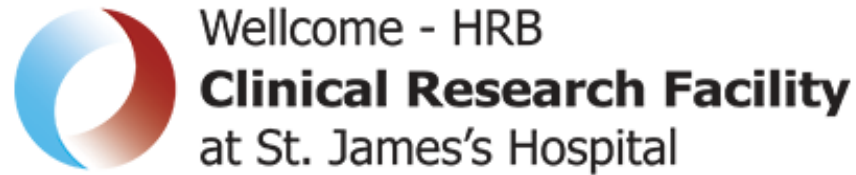
DYNE251-DMD-201

A Phase 1/2 Randomized, Double-Blind, Placebo-Controlled, Multiple Ascending Dose Study Assessing Safety, Tolerability, Pharmacodynamics, Efficacy and Pharmacokinetics of DYNE-251 Administered to Participants with Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping

- First-in-Human Global Study
- MAD -Multiple Ascending Dose Study followed by a long-term extension period
- Expected to enrol approximately 46 ambulant and non-ambulant boys with DMD
- Ages 4 to 16 and have mutations amenable to exon 51 skipping therapy



Opportunity to collaborate



Combining CHI CRC paediatric neuromuscular clinical research expertise with
SJH CRF early phase trial experience



Research & Innovation
Clinical Research Centre



Welcome - HRB
Clinical Research Facility
at St. James's Hospital



Medical Team
Neurosurgery



Physiotherapy



Imaging



Laboratory



Pharmacy



Research Nursing

Clinical Pharmacologists

Cardiology



COMMUNICATE
TRAIN | DEVELOP | SUSTAIN



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THANK YOU



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