



Great Ormond Street
Hospital for Children
Research and Innovation

Research Hospital

Bridging the Gap Between Research & Clinical Care

Lorraine Hodsdon

Head of Nursing & Patient Experience Research & Innovation

Waffa Girshab

Research Hospital Transition Lead Nurse

Laura Chiverton

Senior Research Nurse



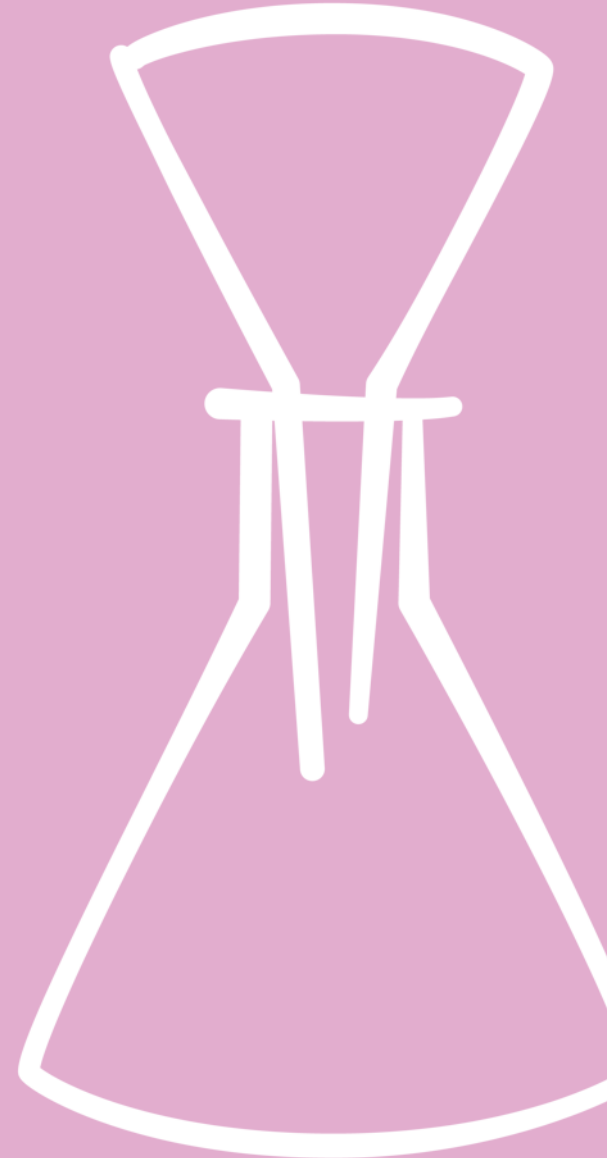


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Aim

- Setting the scene
- Research Hospital Transition Initiative
- Areas of Impact
- Highlights and Future Plans



GOSH is a Research Hospital

Research is fully integrated into every aspect of the hospital, to improve outcomes for our patients and the working lives of our staff.

“...A research-based organisation has a culture of learning...
...research-based organisations tend to have better patient outcomes and patient experience..”



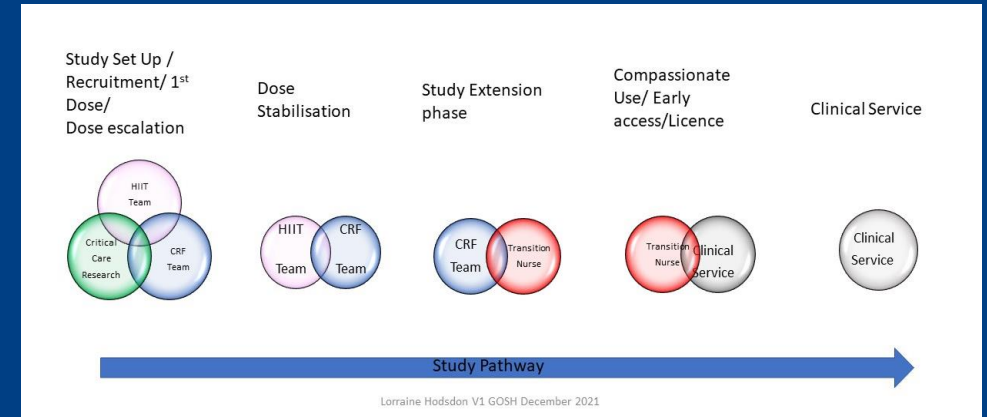
Accelerate
translational
research and
innovation to save
and improve lives

Research Hospital Initiative

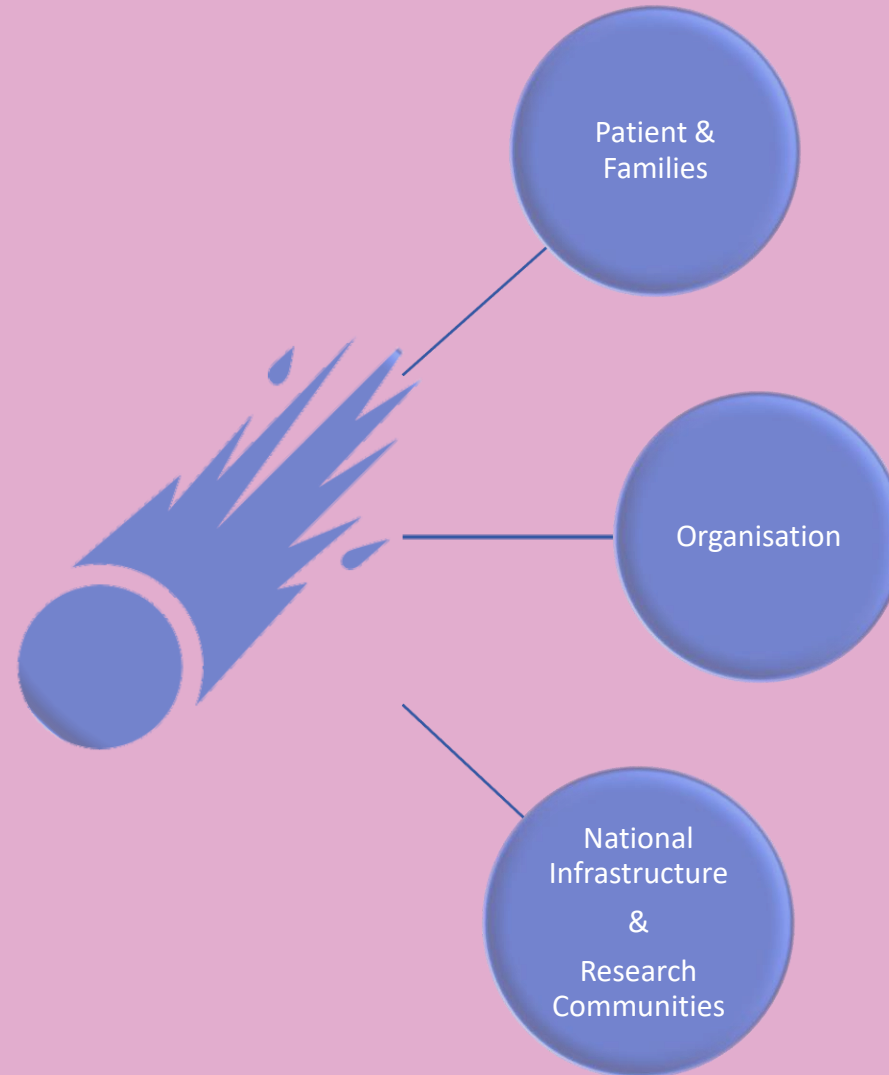
- Build research capacity and capability across the organisation through targeted outreach research leadership and education
- Bridges the skills and cultural gap between research and clinical care, supporting the transition of these services into clinical care and embedding them within the speciality
- To increase opportunities for research participation for all patients by improving our paediatric-to adult research transition pathways.

- Works closely with the Research matrons, research Advanced Clinical Practitioner and directorate matrons to assess the current and future portfolio pipeline
- Identifying studies to transition into the clinical service, being delivered by the ACP or clinical nurse specialist teams with outreach research support
- Opens up capacity within the dedicated research areas to deliver early-phase high intensity research that requires a dedicated research environment
- After successful transitioning many of the compassionate-use programs can eventually move to a home-dosing model, facilitated by the lead nurse and the CNS service, providing further improved and streamlined patient pathways and increasing research access for patients

Research Hospital Transition Model



Research Hospital Transition Impact



Patient & Family Impact

Zain and Zuniarah's Story

- Clinical trial and early access programme for drug X
- Conduit between research care and ward based early access programme = advocating for seamless care
- Collaborative guidelines developed
- New practice pathways embedded into clinical services

'isn't I anything I could think of that didn't go well for me the kids are doing fantastic ...I leave happy knowing that the treatment and care they are getting it's just so fantastic'



Background

- Duchenne Muscular Dystrophy (DMD) is caused by a mutation in the gene that makes dystrophin (a protein). Dystrophin is important for protecting muscles from stress and damage during activity. If a person has DMD, their body is not able to make enough working dystrophin to protect their muscles.
- Symptoms of DMD may be present during the first year of life, but diagnosis is usually made between the ages of 3 and 5 years. The risk of having a child with DMD is about 1 in every 5000 male births.
- Great Ormond Street Hospital have delivered several early phase Sarepta Therapeutics clinical trials since 2014. Leading to the later phase extension studies.

May 2021

Sarepta Therapeutics announced that there would be no extension to SKIPE. Access to Casimersen and Golodirsen would be available through a post-trial access scheme (PTA).

July 2021

Sarepta Therapeutics announced that Baby SKIP clinical trial would be terminating early and that access to Etipirsen will be available via PTA

Sept/Dec 2021

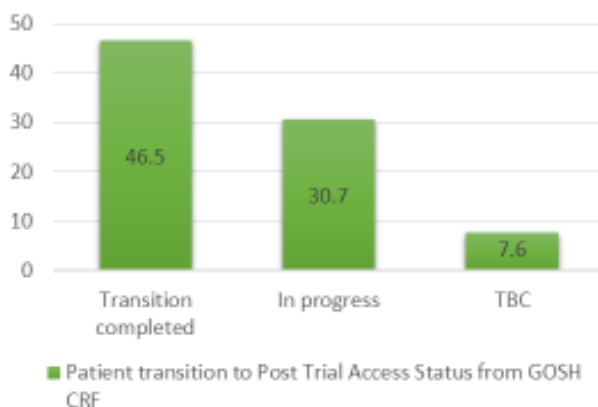
All Baby Skip patients to be transitioned off study by September 2021. All Essence patients to transition off study by December 2021

Transition Progress & Methodology:

- Discussions with sponsors to understand and plan timelines for patients to transition from clinical trials. Following this initial discussion, we identified the local hospitals of patients and initiated contact whilst ensuring all key stakeholders were involved.
- We supported the local hospitals with the governance process for approval of PTA programmes.
- Worked with local hospitals delivering teaching and supporting with clinical and logistical procedures for prescribing and administering the unlicensed medication and setting up.



**Patient transition to Post Trial Access
Status from GOSH CRF**



Family

- Uncertainty surrounding studies ending is difficult for families.
- Happy that sponsor are still providing access to drug.

Local Hospital

- Uncertainty around length of programme. Difficult to plan for.
- Support provided from CRF has made set up easier.
- Once set programme runs smoothly.
- Resource impact on local hospital

CRF

- Managing family/local hospitals expectations on PTA
- Ensuring PTA is set up to meet timelines and ensure no doses are missed.
- Good experience of working collaboratively with sponsor and local hospitals.

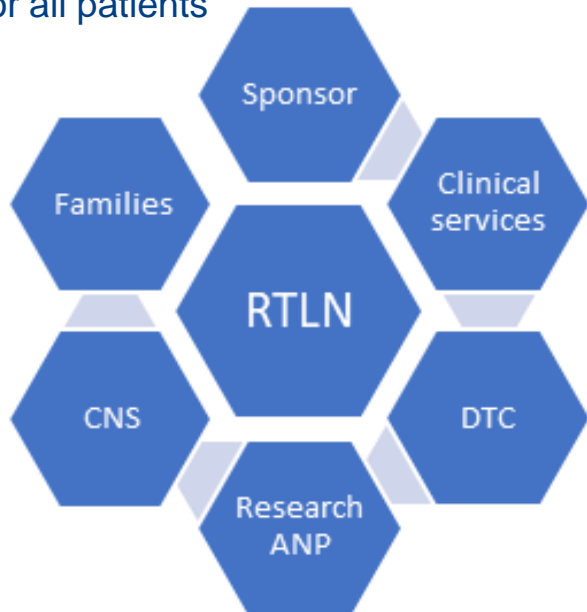
Conclusion and Next steps:

- The CRF nurses alongside the Research Transition Lead Nurse to initiate investigator meetings with the sites across the UK and acted as a lead point of call for Sarepta Therapeutics.
- This helped to understand the distribution of patients and their local hospital and enabled a co-ordinated approach to support a smooth and safe transition of patients to their local hospitals.
- We have successfully transitioned six patients to their local hospitals.
- key the benefit of working collaboratively as a UK to make sure that we planned a co-ordinated approach and local hospitals did not feel overwhelmed.
- recommendation from our experience is to have early discussions with local hospitals when recruiting patients to clinical trials and plan for the possibility of compassionate use in principle. Highlighted the importance exit strategy planning for research patients.

Patient & Family Impact

Ishaan's Story

- Example of a trial life cycle
- Obtained MHRA approval
- Submitted for NICE approval.
- 12 patients at GOSH accessed scheme
- Clinical guidelines created
- Ward staff training/support from RTLN/ANP
- Homecare set up for all patients

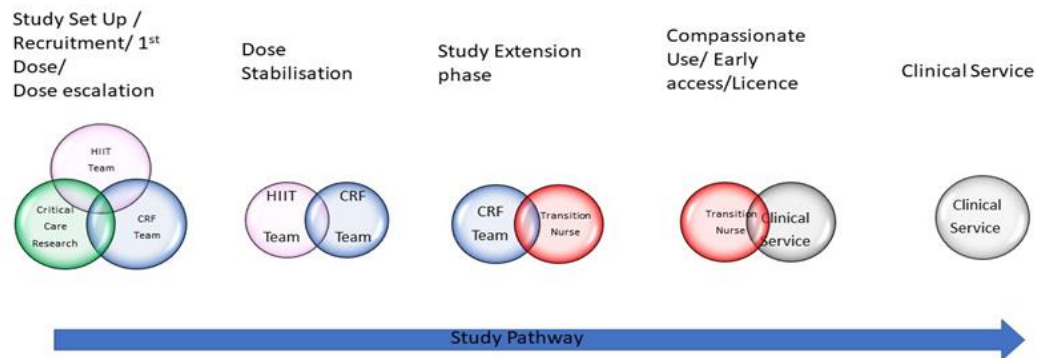


“I am happy, I can still have my treatment and even better that I can do it at home”



Organizational Impact

Hannah's Story



Lorraine Hodsdon V1 GOSH December 2021

'And thank you for being always ahead of what needed for these patients'





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National Infrastructure Impact

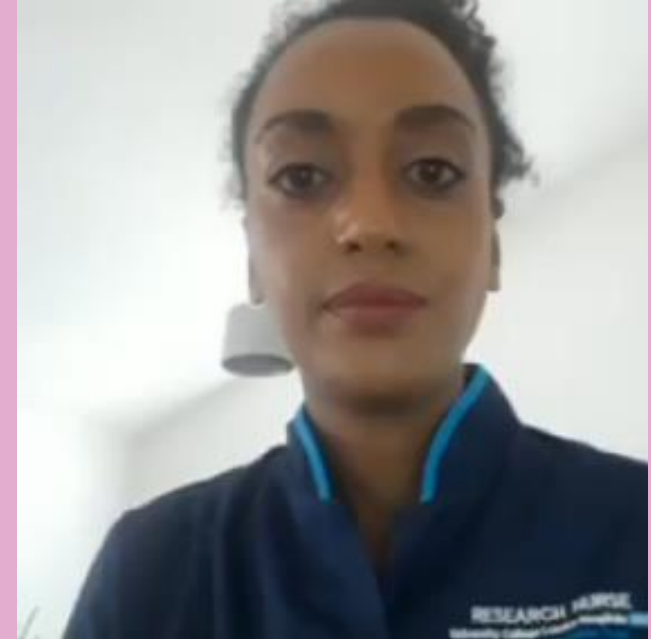
- Collaborations beyond GOSH
- Builds research capability and capacity nationally
- Working with industry to embed new treatments into clinical services

'I cannot begin to express my appreciation for all the assistance and guidance you have provided to all the UK sites to this point. I would love to join the call next week to discuss transitions patients'
post trial access director –pharma



Research Community Impact

- Pediatric to Adult transition research centers
- Equity of access for all cohorts
- Support research in underserved populations
- Shares best practice models



'Early discussion and handover meant that we could plan a smooth transition for this patient with complex need and were able to safely accept the patient'



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Highlights

- 11 Studies transitioned into clinical care
- 28 Patients transitioned from trial ending onto post trial access.
- 16 non research patients given early access to medication in clinical services
- Research transition embedded in Trust transition pathway
- Compassionate use SOP developed
- Partnership working across boundaries re study exit strategies
- Positive organisational research culture step change
- Transition working group

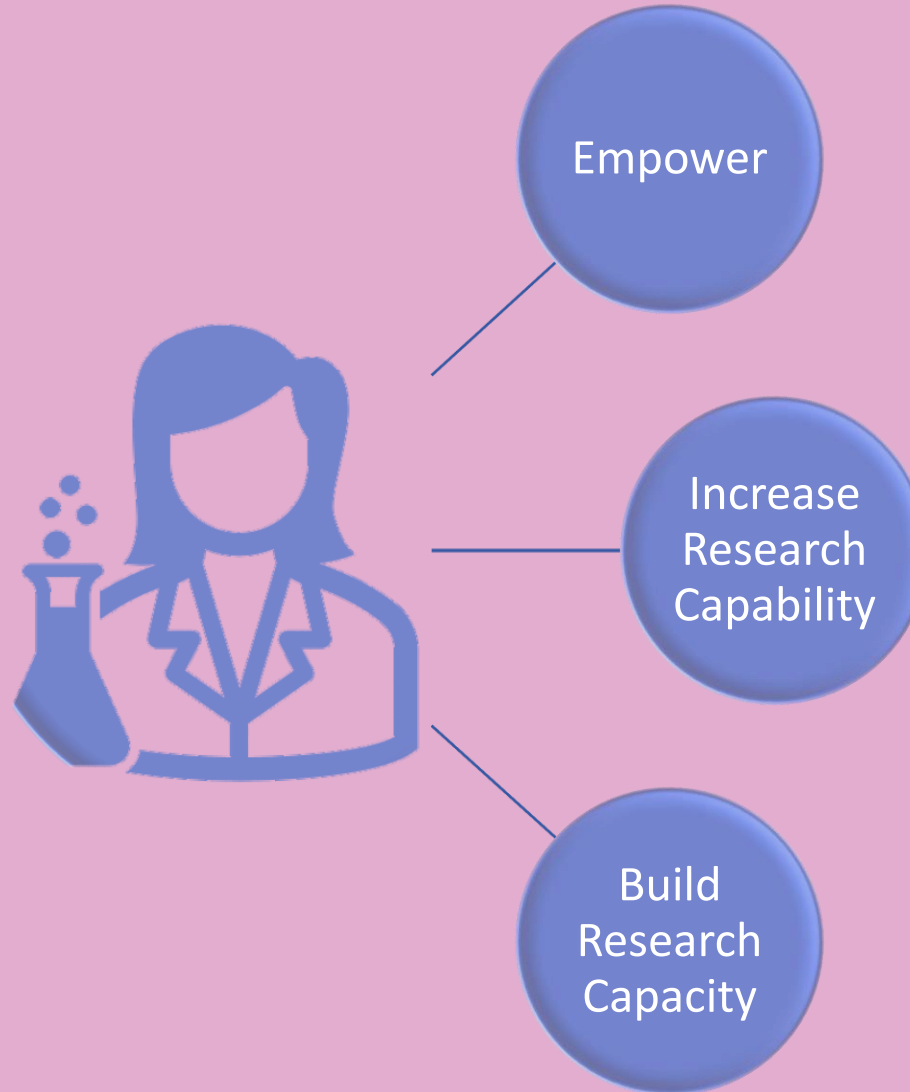
Future

- Research infusion lounge
- Homecare pathways
- National research transition study days
- Strengthen national collaborations

Summary

Research Hospital Transition is
Core to Care

Our unique patient population
means we are well placed to lead
the transition of paediatric early
phase research into clinical
services



Thank you



Researchrocks