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# Driving forward research in patients with a single ventricle

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Little Hearts Matter Medical Symposium, Monday 30<sup>th</sup> September 2024

# Single ventricle research

- Lack of high-quality, robust evidence to inform clinical decision-making
- Few practice-changing clinical trials in children and adults
  - Most trials are small, single-centre studies, low value → no clinical impact
  - Even larger international trials often struggle to recruit, compromise on outcomes, and have neutral findings
  - Most UK centres have limited experience of leading or participating in clinical trials
- Need for high-quality, multi-centre trials to improve outcomes for patients & families
  - Identify the most important research questions for the wider community
  - Develop the structures & workforce to deliver clinical trials → improve patient care

# Single ventricle priority setting study (2018)



- Brought together stakeholders to establish national priorities for SV research <sup>1</sup>
- Workshop held in April 2018, agreed 30 priorities across 9 areas:
  - Associated co-morbidities
  - Brain & neurodevelopment
  - Exercise
  - Fontan failure
  - Transplantation, mechanical support & novel therapies
  - Heart function
  - Living with a single ventricle heart condition
  - Management of the well-functioning Fontan
  - Surgery & perioperative care
- Provided a focus for clinicians, researchers and funders to address the most important research questions in single ventricle heart conditions

<sup>1</sup> Drury NE et al. *Cardiol Young* 2019.

# Research priorities in single-ventricle heart conditions: a United Kingdom national study

## Original Article

### \*Collaborators:

Patients and parents: Emma Adams, Isabel Bamber, Kerry Crofts, Sally Evans, Donna Giles, Wendy Knowles, David Simpson, Anthony Smith, Patrick Smith; Birmingham Children's Hospital: Mr David J Barron, Mr William J Brawn, Dr Rami Dhillon, Dr Haifa Eltayeb, Ms Natasha E Khan, Justine Kidd, Julie C Menzies, Prof Kevin P Morris, Jacqui Nisbett, Nikki Rehman, Dr Anna N Seale, Dr Oliver Stumper; Bristol Royal Children's Hospital: Mr Andrew J Parry; Evelina London Children's Hospital: Dr Hannah R Bellsham-Revell; Great Ormond Street Hospital, London: Mr Ben Davies; Little Hearts Matter: Lexie Katsatis; Newcastle University & Freeman Hospital, Newcastle: Dr Bill Chaudhry, Dr Louise Coats; Queen Elizabeth Hospital Birmingham: Tom Gaffey, Nichola Pope, Mr Ivan Yim; The Somerville Foundation: John Richardson; University Hospital Southampton: Dr Catherine L Mercer; University of the West of England: Dr Lyvonne N Tume; University of Worcester: Dr Kerry L Gaskin.

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### Abstract

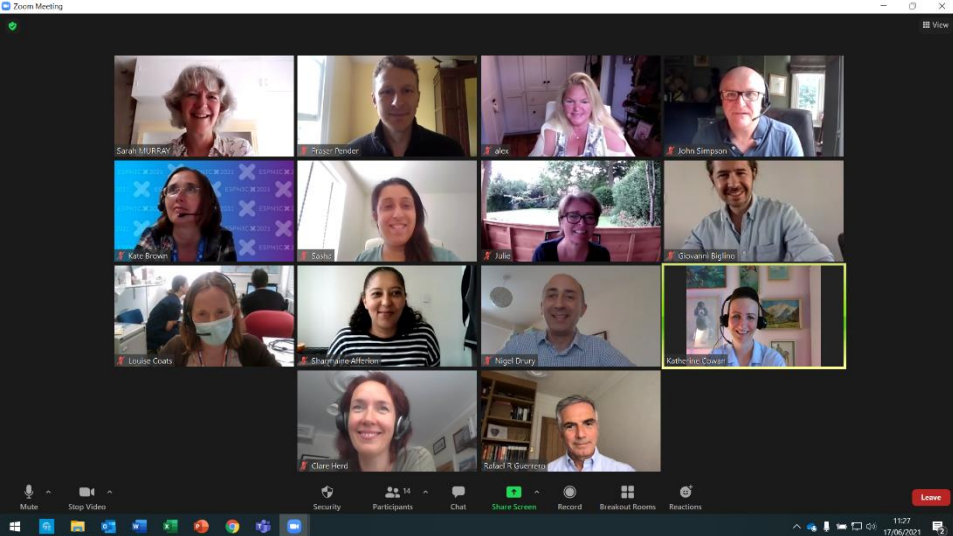
*Objective:* To bring together stakeholders in the United Kingdom to establish national priorities for research in single-ventricle heart conditions. *Methods:* This study comprised two surveys and a workshop. The initial public online survey asked respondents up to three questions they would like answered for research. Responses were classified as unanswered, already answered, or unable to be answered by scientific research. In the follow-up survey, unanswered questions were divided into categories and respondents were asked to rank categories and questions by priority. A stakeholder workshop attended by patients, parents, healthcare professionals, researchers, and charities was held to determine the final list of research priorities. *Results:* A total of 128 respondents posed 344 research questions, of which 271 were classified as unanswered, and after removing duplicates, 204 questions remained, which were divided into 20 categories. In the second survey, 56 (49.1%) respondents successfully ranked categories and questions. A total of 39 participants attended the workshop, drawing up a list of 30 research priorities across nine priority categories. The nine priority categories are:

# JLA CHD Priority Setting Partnership (2021-22)



- National process to identify and prioritise unanswered research questions
  - PSPs provide an equitable mechanism for prioritising research
  - Brought together patients, parents, charities and clinicians
  - Scope: diagnosis, treatment and outcomes of CHD throughout life
  - LHM was a partner organisation → publicise surveys, represented at workshops
- Two Top 10 lists of priorities → one child/antenatal, one adult <sup>2</sup>
  - Some priorities appear on both lists → 14 unique priorities:  
4 child/antenatal, 4 adult, and 6 throughout life
  - Provides a platform for conducting the research that matters most
  - Many are directly relevant to patients with a single ventricle heart condition

<sup>2</sup> Drury NE et al. *Open Heart* 2022.



**openheart** Research priorities in children and adults with congenital heart disease: a James Lind Alliance Priority Setting Partnership

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# JLA CHD Priority Setting Partnership (2021-22)



- How can the **longevity of the Fontan circulation** be prolonged and the impact of complications (e.g. liver, protein-losing enteropathy, renal, endocrine, fertility) be reduced? – adult #2, child #9
- What is the impact of living with CHD on **mental health** in children/adults and how can this be improved through access to psychological support and other therapies? – adult #3, child #6
- How can technology be used to deliver **personalised care** and improve outcomes in CHD (e.g. artificial intelligence, 3D printing, genomics, stem cells, organ regeneration)? – adult #4, child #5
- What is the impact of living with CHD on **quality of life** in children/adults and how can this be improved? – adult #9, child #7
- How can **damage to organs** (e.g. heart, brain, lung, kidney, bowel) during heart surgery in children with CHD be minimised to reduce complications, especially in those who require multiple operations? – child #1
- How can the indications, timing of referral, and outcomes of **transplantation** and long-term mechanical support in adults with CHD be improved? – adult #8



# Transforming collaborative research

A national strategy to address the James Lind Alliance priorities for children and adults with congenital heart disease

July 2023



In partnership with:



## FOREWORD

Congenital heart disease (CHD) is the most common type of birth defect, affecting 13 children born every day in the United Kingdom (UK).<sup>1</sup> It has a lifelong impact and with improved outcomes during childhood, there are now more adults than children living with CHD in developed countries.<sup>2</sup>

It therefore may be surprising that there is a lack of evidence to inform clinical decision-making in both children and adults with CHD. The Cochrane Library contains fewer than twenty reviews on CHD topics,<sup>3</sup> and there is a need for high-quality, multi-centre clinical trials that answer important questions to improve the daily lives and outcomes of those affected.<sup>4</sup> The British Heart Foundation (BHF) identified that 'we urgently need research breakthroughs to ensure survivors [of CHD] lead longer and healthier lives'.<sup>5</sup>

With national commissioning of specialised services and lifetime follow-up, access to National Institute for Health and Care Research (NIHR) infrastructure, well-developed clinical trials units, and the recently established All-Island CHD Network, the UK and Ireland should be an ideal environment in which to conduct world-leading CHD clinical research. However, the last two decades have been challenging times for CHD services in the UK, following the Bristol Royal Infirmary Inquiry,<sup>6</sup> coupled with a lack of clarity regarding the future configuration of national services, leading to an environment of uncertainty and limited collaboration between centres. In recent years, following publication of the British Congenital Cardiac Association (BVCCA) statement on 'Multi-centre working',<sup>7</sup> this has begun to change, with several prospective multi-centre studies and there is enthusiasm amongst families, healthcare professionals and research funders to develop more collaborative research.

Working with the James Lind Alliance, we brought together patients with lived experience of CHD, their families, charities, and healthcare professionals in the UK to form the national Congenital Heart Disease Priority Setting Partnership.<sup>8</sup> Through a shared decision-making process, we determined two Top 10 lists of priorities for CHD research, one child/antenatal and one adult. Remarkably, six of the priorities were present on both lists, leading to 14 distinct clinical priorities: four child/antenatal, four adult and six throughout life.

This document sets out these research priorities and describes a national strategy to address them through collaborative research, endorsed by both professional bodies and national charity partners. First, to establish a UK and Ireland network for multi-centre research, focusing on clinical trials and other studies that have the potential to change clinical practice. Second, to set-up a national CHD Patient and Public Involvement (PPI) group, comprising engaged patient, parent, and charity members with lived experience or affected by CHD, to contribute through all stages of the research. Third, to develop specific working groups of clinicians, researchers, and PPI members, to address each of the priorities. And finally, to learn from the experiences of others in conducting multi-centre CHD research and translating research priorities into funded clinical studies.

The priorities provide a platform for conducting the research that matters most, whilst the strategy outlines a structure through which they can be translated into research questions and funded studies. I believe that together these present a unique opportunity to transform collaborative CHD research in the UK and Ireland for the benefit of the whole community.



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# Congenital Heart Research Network



British Congenital  
Cardiac Association



SCTS  
Society for Cardiothoracic Surgery  
in Great Britain and Ireland

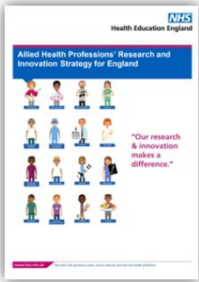
- Collaborative network for multi-centre studies in UK & Ireland
- Impactful clinical trials and other studies → change practice → improve outcomes
- Framework for Investigators to develop and lead studies
  - Structures approved by BCCA Council, incl. Executive and Scientific committees
  - Developing Manual of Operations, including clear & agreed terms of reference
  - National CHD Patient and Public Involvement (PPI) group, including charities
  - Clinical Study groups: develop priorities → funded clinical studies
  - Affiliated NMAHP National Research network, first meeting last week
- Training: enhance research skills of CHD workforce, especially on clinical trials
- Planning a launch event in spring 2025, pending funding → watch this space!

# Jara's PhD work

- Patient with a Fontan circulation leading research
- Exploring issues that are important to her and her peers
- Fits with the national CHD priorities, especially on
  - Quality of life (adult #9)
  - Mental health (adult #3)
- Improve understanding of what life is like for those living with Fontan
- Potential impact
  - Identify areas of need
  - Inform clinical guidelines
  - Advocate for changes in social policy



# Research culture, capacity and capability – NMAHPs



## Phase 1: Discover

- Ascertain baseline – research culture, capacity, capability of NMAHPs
- <https://app.onlinesurveys.jisc.ac.uk/s/bcu/research-culture-capability-and-capacity-chd>

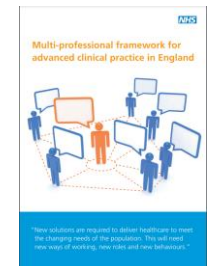


## Phase 2: Build

- Implement programme of interventions to support development of capability and capacity for NMAHPs
- Prioritise areas of research



## Phase 3: Sustain – driving forward research



# Summary

- Priorities provide platform for conducting the research that matters most
  - Many of the JLA priorities relate to children & adults living with Fontan circulation
- Strategy outlines structures to translate priorities → research questions → funded studies with potential to change clinical practice → improve patient care
- Together, unique opportunity to transform collaborative CHD research in UK & Ireland
- Funding opportunities include:
  - BHF-CRC research development grants e.g. systematic review
  - NIHR rolling call *specific* for JLA PSP-identified priorities: HTA, EME, HSDR, PHR
  - BHF Clinical Study grants – strategy identified CHD research as ‘urgent’ priority<sup>3</sup>

<sup>3</sup> British Heart Foundation. *Our strategy to 2030*.

**Thank you!**

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