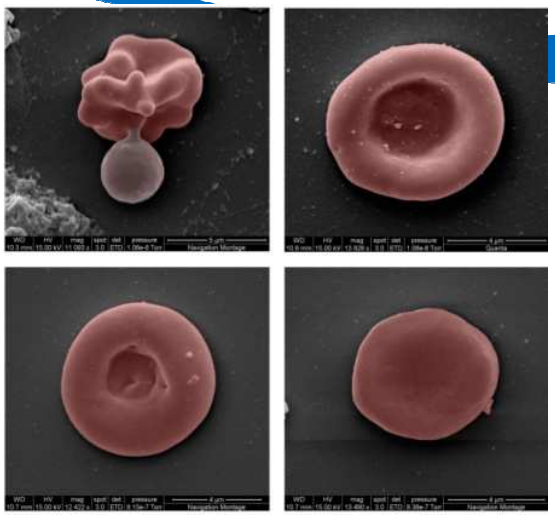
NHS
Blood and Transplant

INTERVAL



Planet-2

COMPARE

TREATT

NHSBT's R&D Strategy: 2015 – 2020

Improving outcomes for donors and patients

Dr Nick Watkins BSc DPhil MBA
Assistant Director – R&D

Caring Expert Quality

NHS
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- PhD University of York (1996)
- Joined Prof Ouwehands' group in 1995
- Over 100 peer-reviewed publications
- Secured over £40M R&D Funding
- MBA University of Cambridge (2008)
- 2 years as Safety Programme Coordinator
- Head of Cambridge Centre since 2005
- Appointed AD-R&D June 2011
  - Responsible for delivery of R&D Strategy

Who we are , what we do

NHS Blood and Transplant

Blood Supply

1.7 million donations

900,000 donors

21,000 + sessions

Team of 3,000

£285m turnover

13% decline in demand over 5 years

Organ Donation & Transplantation

1,281 deceased donations last year

21m registrants on the ODR

4,415 transplants

Team of 420

£71m grant

Diagnostic & Therapeutic Services

12,000 tissue implants

50% UK stem cell transplant market

40% NHS H&I testing market

Team of 750

£65m turnover

Our capabilities – supporting the NHS

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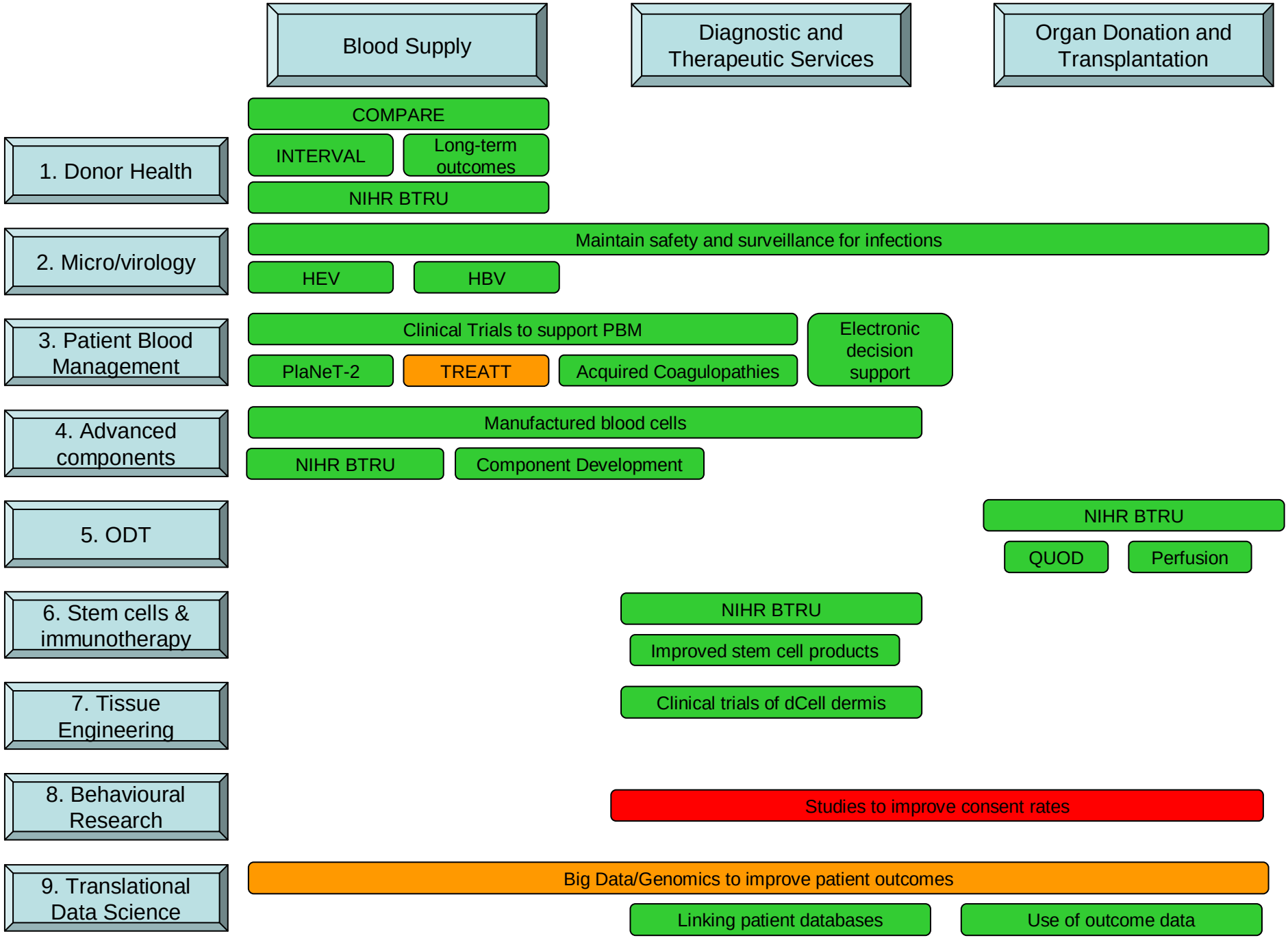
Why does NHSBT do research?

- **To improve outcomes for patients and donors and to improve our services to the NHS**
- Maintains credibility and international reputation – ours is a scientifically-led field
- Attracts medical and scientific talent which benefits teaching and service provision
- Competitive edge
- Investment in R&D continues to be important

NHS Blood and Transplant

Our Strategic intent: 2015 - 2020

- To deliver an innovative and translational R&D programme:
  - through strong academic partnerships;
  - based around our unique capabilities
    - Embedding studies in operational environment
    - Large datasets
- To deliver improvements in donor care and patient outcomes.


Blood Supply
Diagnostic and Therapeutic Services
Organ Donation and Transplantation
1. Donor Health
COMPARE
INTERVAL
Long-term outcomes
NIHR BTRU
2. Micro/virology
Maintain safety and surveillance for infections
HEV
HBV
3. Patient Blood Management
Clinical Trials to support PBM
Electronic decision support
PlaNeT-2
TREATT
Acquired Coagulopathies
4. Advanced components
Manufactured blood cells
NIHR BTRU
Component Development
5. ODT
NIHR BTRU
QUOD
Perfusion
6. Stem cells & immunotherapy
NIHR BTRU
Improved stem cell products
7. Tissue Engineering
Clinical trials of dCell dermis
8. Behavioural Research
Studies to improve consent rates
9. Translational Data Science
Big Data/Genomics to improve patient outcomes
Linking patient databases
Use of outcome data

Working in partnership with four new centres of academic excellence

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Donor Health and Genomics
University of Cambridge
John Danesh/Emanuele di Angelantonio

Iron stores and blood cells
Health consequences of donation
Personalised donation

Organ Donation & Transplantation
University of Cambridge/Newcastle
Andrew Bradley/Andy Fisher

Improve donor management
Assessing organ quality & function
Reducing re-transplantation

£14.4M

Stem Cells & Immunotherapies
University College London
Karl Peggs/Amit Nathwani

Predict high risk GvHD
Re-direct immune cells
Correcting inherited blood disorders

Manufactured red cells
University of Bristol (TBC)
Dave Anstee/Ash Toye

cRBCs as a lead ATMP
Generate a small-scale product
Complementary to BloodPharma

Improving donation practices

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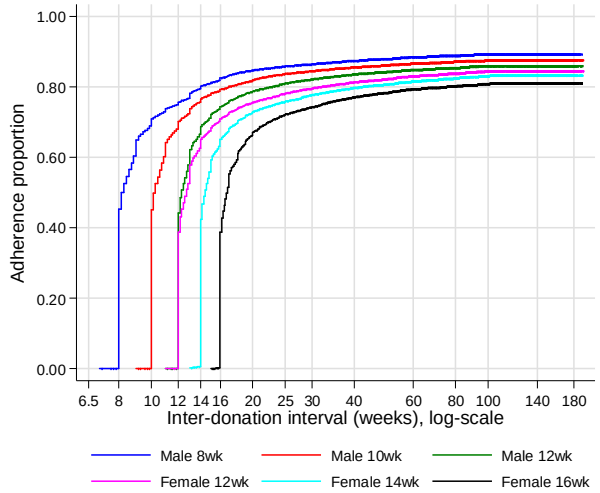
- Personalising donation intervals



- Results expected November

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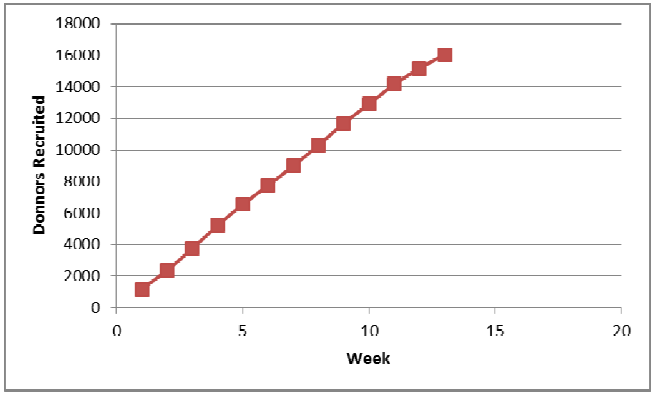
- Hb measurements in 31,000 donors



- Actively recruiting to Phase I

Danesh/Di Angelanonio/Roberts/Ouwehand

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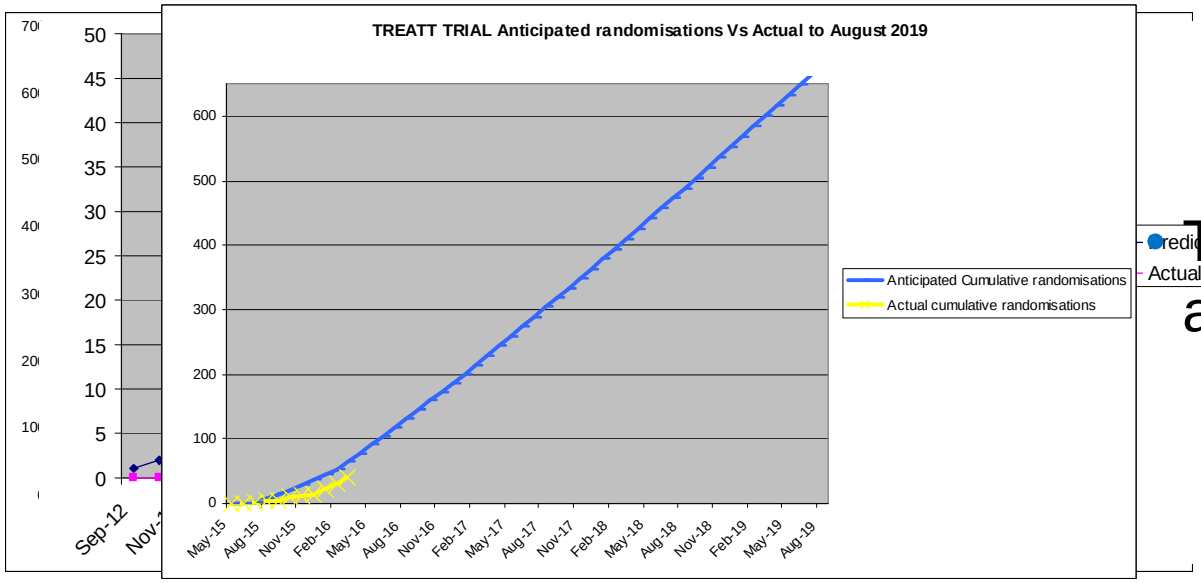
Maintaining blood, tissue and organ microbiological and virological safety

- Recruited a new PI as part of succession planning:
  - Dr Nick Matheson, University of Cambridge
- Working in partnership with Public Health England:
  - Blood Borne Virus Unit, epidemiology team
- Focus on Hepatitis E virus:
  - Large study on frequency in blood donors published;
  - Findings used to inform SaBTO decision making;
  - Current study looking at HEV in transplant recipients
- vCJD assay development and testing activities brought to a close:
  - All affected members of staff were successfully redeployed

Clinical trials to inform patient blood management

- Our clinical trials unit achieved UKCRC registration in 2015



TREATT is up and running

NeITA2 is up and running ahead of recruitment

Collett/Stanworth/Marsh

Advanced blood components: alternatives for patients who are very difficult to match

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Rare Blood diseases
e.g. Sickle Cell
Beta Thalassaemia

Multiple transfusions

Alloimmunity
(making antibodies against transfused blood)

Person becomes increasingly difficult to match for transfusion

1. Manufactured red blood cells (mRBC) would enable NHSBT to provide blood for these individuals
2. Manufactured platelets could have HLA silenced to remove need for matching.

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- **RESTORE: Recovery and Survival of Stem Cell Originated Red Cells**
  - Mini-dose of red cells derived from CD34 pos cells from adult blood vs standard donated RBCs
- **Current status:**
  - Scientific Project Manager appointed
  - Manufacture transfer to GMP (Filton); ATMP licence in progress;
  - Trial Steering Committee established;
  - Patient/Public group established and reviewing documentation;
  - IMPD for MHRA in preparation;
  - Ethics application in preparation
  - Donors being approached- positive response
  - First transfusions planned for 2017

Improving outcomes in organ transplantation

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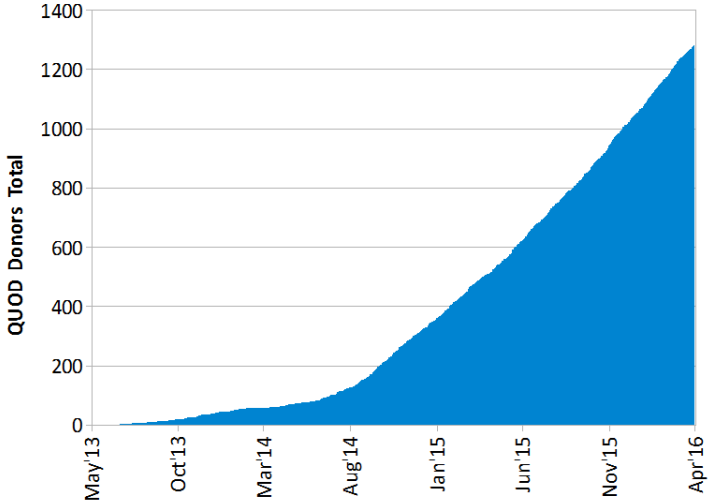
- Over 1,300 donors consented to the QUOD bioresource
- 18 studies have applied for samples from the bioresource:
  - Assessment of a kidney and liver donor histopathology service
  - Reimbursement scheme being developed
- Fast-track allocation scheme in pancreas transplant



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Developing the next generation

- 18 PhDs obtained in 2015
- 7 Clinical Research Fellows
- 2 Academic Clinical Fellows
- 1 Academic Clinical Lecturer
- 5 year junior group leader position recruited in Cambridge - Dr Marloes Tijssen
- Second tenure track post for Bristol in regenerative medicine

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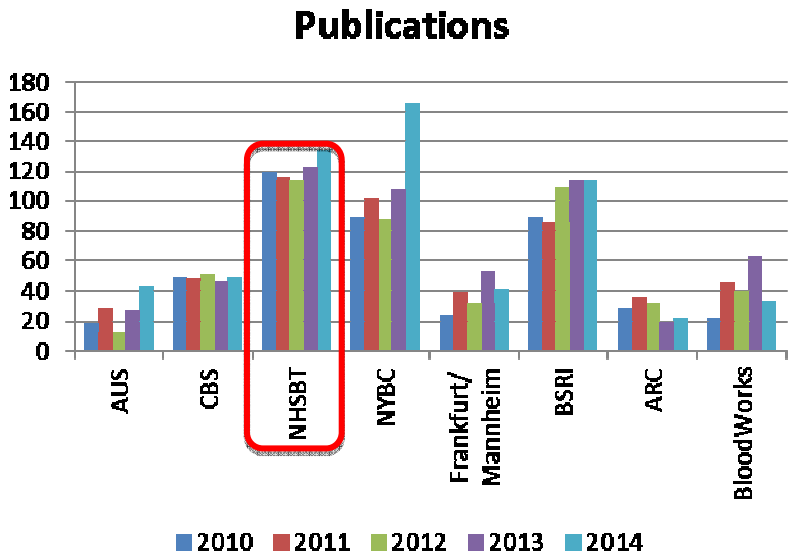
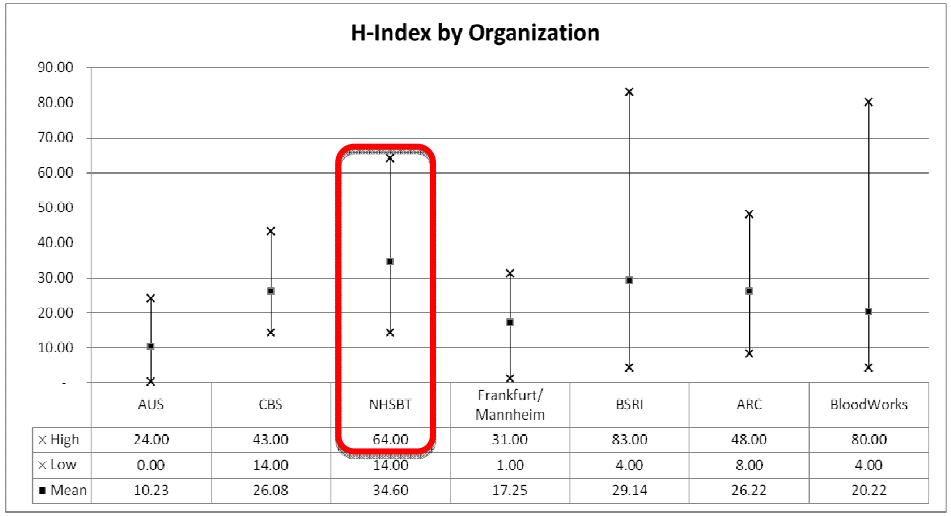
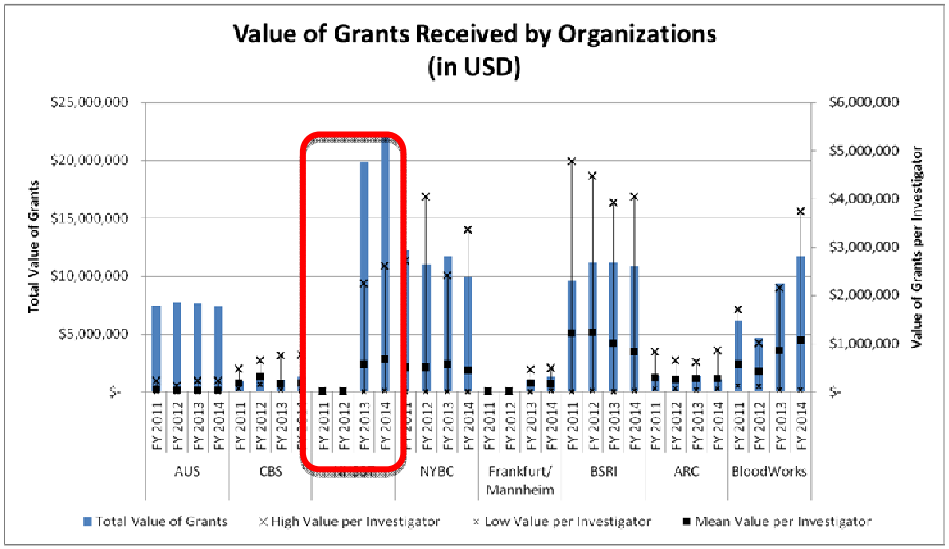
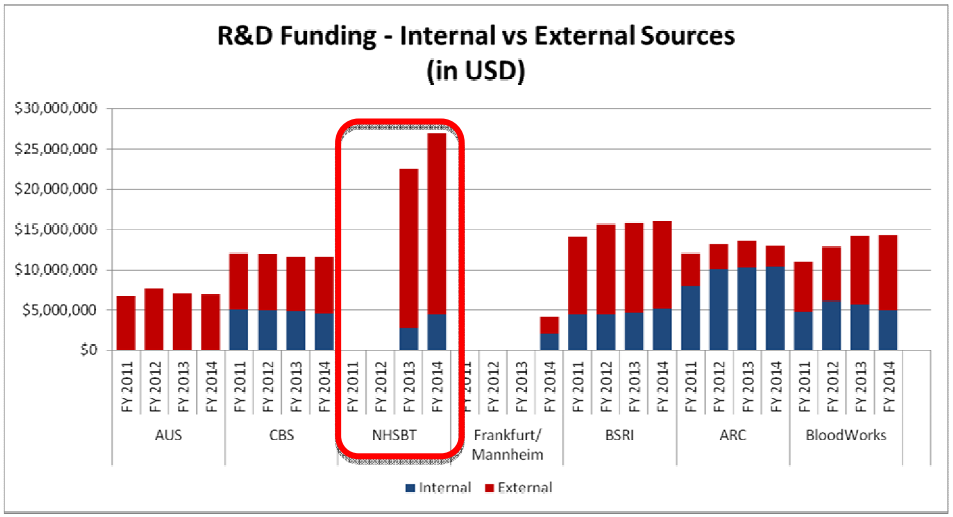
Benchmarking - ABO R&D working group





	AUS	CBS	NHSBT	Frankfurt/ Mannheim	BSRI	ARC	BloodWorks
× High	24.00	43.00	64.00	31.00	83.00	48.00	80.00
× Low	0.00	14.00	14.00	1.00	4.00	8.00	4.00
■ Mean	10.23	26.08	34.60	17.25	29.14	26.22	20.22





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Translating R&D outputs into service

- Tissue Development Laboratory:
  - Temperature validation of eye transport boxes
  - Reduced cryomedium exposure time during skin processing
  - Temperature validation of kidney transport boxes

- Component Development Laboratory
  - New process for manufacture of cryoprecipitate (Saving time/cost)
  - Extension of the shelf-life of thawed FFP (Reduced wastage)
  - Remanufacture of exchange transfusion units (Reduced wastage)

- COPE trial of hypothermic kidney preservation supported routine use of machine perfusion
- Implemented next generation HLA sequencing for better graft matching and to type all adult and cord blood donors (H&I Service Development)
- Rare inherited platelet disorder diagnosis using next generation sequencing
- Diagnostics Development and IBGRL moved into DTS

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Our R&D Strategy will ensure we provide innovative and advanced treatments to those who depend upon our products and services

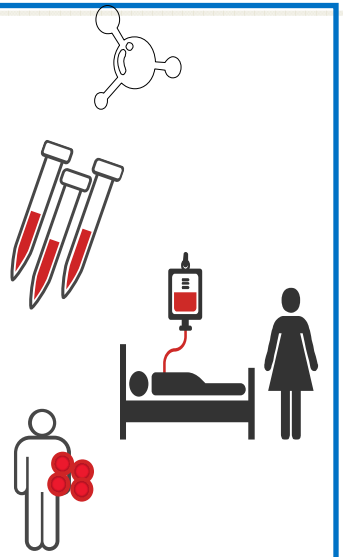
www.nhsbt.nhs.uk/research-and-development

Research & Development

NHS Blood and Transplant

Increasing safety & improving outcomes

- **Next Generation Sequencing** for HLA – maximising efficiency of stem cell donors and donations
- Research in microbiology & virology to **maintain blood, tissue and organ safety**
- Clinical trials to support **Patient Blood Management**
- Development, assessment and clinical delivery of innovative **Regenerative Medicine** based therapies



Supporting R&D through our unique position

- INTERVAL Study - research with 50,000 blood donors to develop **personalised donation practices**
- Quality in Organ Donation (QUOD) – **increasing numbers and quality** of deceased donor organs for transplantation

INTERVAL

Headlines

NHS Blood and Transplant

- Renewed 5 years NIHR funding to 2020: £14.4M
  - Established four Blood and Transplant Research Units;
- Recruited to three leadership positions in Cambridge:
  - Dr Simon Mendez-Ferrer - Reader in Stem Cell Biology;
  - Dr Nick Matheson - Senior Research Fellow in Virology;
  - Dr Marloes Tijssen - Research Scientist – Programme Lead;
- £5.1M funding from external funders:
  - £1.9M from HTA/NIHR for a clinical evaluation of dCELL dermis in diabetic leg ulcers;
- 153 manuscripts in international scientific journals;
- Clinical trials unit achieved UK Clinical Research Collaborative registration
- Recruited to clinical studies from operational environment:
  - INTERVAL concludes at the end of June 2016
  - 1,300 organ donors have provided samples for QUOD
- Planning for first-in-man trial of manufactured red cells (RESTORE)

Our Goals (2015 – 2020)

NHS Blood and Transplant

1. To establish and ensure delivery of NIHR Blood and Transplant Research Unit objectives through partnership working

2. To enhance our programme of research in transfusion/transplantation microbiology and virology to maintain blood, tissue and organ safety

3. To deliver clinical trials to support patient blood management

4. To strengthen our position in the development, assessment and clinical delivery of regenerative medicine based therapies

5. To establish a Behavioural Research programme to identify behavioural change interventions which significantly increase donation and consent rates

6. To establish a Translational Data Science programme to build and exploit big data resources that deliver improvements to our services

7. To provide facilities and resources to support an innovative research programme

8. To ensure that our workforce have the skills and expertise to deliver the R&D Programme