Worldwide Involvement of Children in Clinical Research

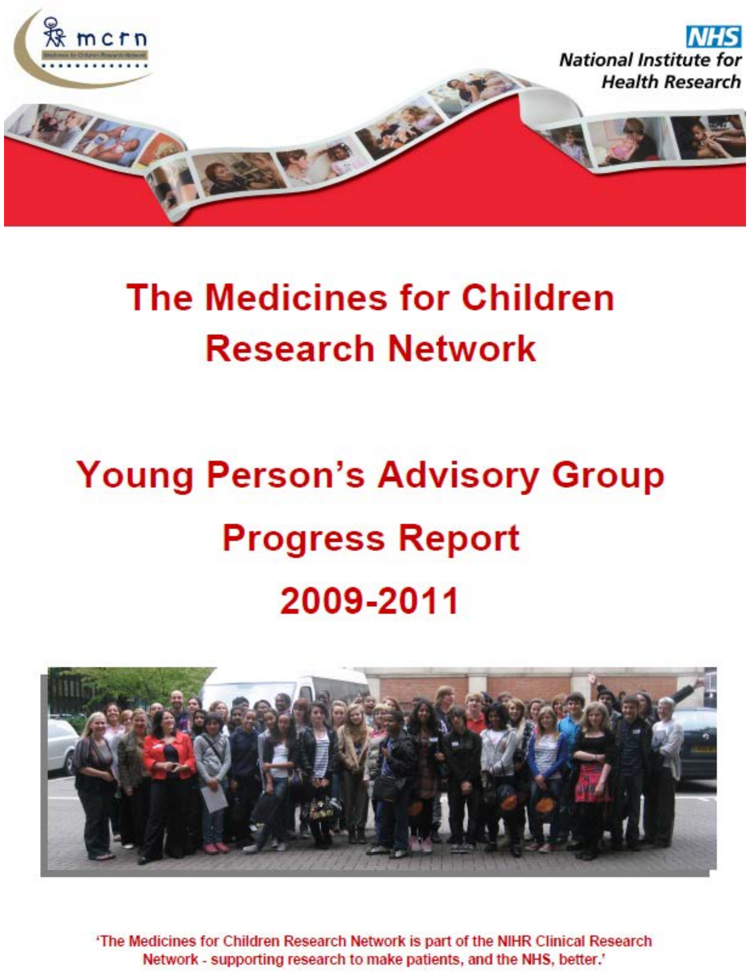
Pamela Dicks
Jenny Preston
Charlie Thompson

Enpr-EMA Annual Workshop, June 2014

Agenda

- Overview of KIDS program in USA
- Vision for an international network
- Discussion

Background (UK Experience)



mcrn

NHS
National Institute for
Health Research

The Medicines for Children Research Network

Young Person's Advisory Group

Progress Report

2009-2011

'The Medicines for Children Research Network is part of the NIHR Clinical Research Network - supporting research to make patients, and the NHS, better.'

Young people help researchers to "get real"

Clinical research is evolving. Increasingly, there is pressure on the research community to ensure that treatments are relevant and effective in daily life. This is driving more researchers to seek the input of service users in the development of their work, which is leading to a transformation of the cultural and regulatory environment for children's research.

▲***The Young Persons' Advisory Group** is growing in size and influence*

The NIHR Medicines for Children Research Network (MCRN) has been at the forefront of patient and public involvement in research for some time. In 2006, the Network started its first Young Persons' Advisory Group in Liverpool. Since then, four more groups have been established in London, Nottingham, Birmingham and Bristol.

The initial remit of these groups was straight forward: to engage young people with research and to work in partnership with, and offer support to, researchers. Jenny Newman, NIHR Medicines for Children Research Network Consumer Liaison Manager, explains how this role has evolved:

"We set up the group to provide a forum for young people to learn about, and comment on, various aspects of the research cycle from the identification of research questions to the dissemination of research findings. We are now working with national governing bodies and helping to remodel the guidance they provide to researchers to help them design and deliver ethically robust research for children, as well as support researchers in the design and deliverability of their studies."

> "Young people... want to know what the study will mean to them."

Parents are able to consent for children to take part in clinical research if they are under 16 years of age. But researchers have to gain the assent of children too, which is why they receive guidance on how to produce materials to help young people understand a study.

The National Research Ethics Service (NRES) is responsible for producing this guidance. When they decided to review their materials, NRES approached the Medicine for Children Research Network about working with the Young Persons' Advisory Group:

"We conduct training for researchers in the area of ethics guidance and over the last five years the MCRN's Young Persons' Advisory Group has become an important part of our meetings", explains Dr Hugh Davies, consultant paediatrician and Research Ethics Advisor at NRES. "As a result, we wanted their input in our guidance review, which has led to a major statement of how we should approach children."

Group members felt that NRES guidance was producing study materials that failed to meet their needs. Holly Lamden is 18 years old, and a member of the Liverpool Young Persons' Advisory Group:

"A lot of the materials we see are highly formulaic. They are clearly designed to tick legal and governmental boxes, but they produce assent forms that are 15 pages long. An eight year old is not going to read this. Researchers need to make a distinction between child and adult studies. Paediatric studies need to stand alone."

Holly and her fellow Young Persons' Advisory Group members left NRES in no doubt that researchers had to work much harder to seek children's assent to clinical research. Dr Davies continues:

"We learnt that one of the guiding principles for producing information for young people is that they want to understand research and how it will impact on their lives. They want to know what the study will mean to them."

The result is that NRES will be incorporating the Young Persons' Advisory Group's feedback into a section of their guidance designed specifically to help researchers understand how to write and produce study materials for children.

This is a significant success for the Medicines for Children Research Network and a major step for the broader paediatric research community. The Network has worked hard to place young people at the heart of this community and the NRES collaboration shows this hard work is bearing fruit.

This shift in culture is further evidenced by the number of life-sciences organisations now approaching Medicines for Children for support from the Young Persons' Advisory Group. The Group has worked with 10 life-sciences organisations, but half-a-dozen of them have approached the Network in the last six months.

Mitsubishi Pharma Europe Ltd is one of the organisations that has benefitted from partnering with the Young Persons' Advisory Group. It spent over four months developing patient resources for its first paediatric study, but made significant progress when its representatives spoke to the Group. Anna Muroni is a Clinical Research Scientist at Mitsubishi Pharma Europe Ltd:

▲***Holly** feels young people have an important role to play in the development of children's research*

"We benefitted from a range of advice from the Group. Based on their advice we developed a colourful and child-friendly flip-chart to accompany the assent form.

"They also advised us to reduce the length of the form itself… we made a number of alterations including simplifying the assent text and increasing its font size. This advice contributed to us gaining ethics approval at our second attempt."

Mitsubishi Pharma Europe Ltd is now reviewing its processes, with a view to producing paediatric patient information, only after meeting with service users. But the Medicines for Children Research Network is keen to get young people involved in the conception of the research question itself.

Dr Enitan Carrol is a Reader in Child Health and Consultant in Paediatric Infectious Diseases at the University of Liverpool Institute of Child Health. She recently used a workshop with the Young Persons' Advisory Group to determine whether the research question she was proposing was valid. She feels that, in an increasingly competitive environment, this input can prove vital:

"It's powerful that the people experiencing a diagnostic device have helped to shape it. Their involvement makes a study more competitive because it provides us with perspectives that we can't find anywhere else. It makes the product more likely to succeed and helps us avoid the development of expensive products that children do not want to use."

Dr Davies agrees that involving children at the beginning of the research process is crucial:

"The research community is concentrating on studies that are relevant and shaping the right question is key to this. The opportunity for genuine influence is limited, which is why it needs to be embedded in the early stages of research development."

With the paediatric research community increasingly focusing on the real-world relevance of research, the influence and impact of the Young Persons' Advisory Group is set to grow. In September this year it is holding its first national event for the life-sciences industry in which a wider range of organisations will gain an insight into how young people and families can have a positive impact on the development of clinical research.

www.mcrn.org.uk

Watch a Young Persons' Advisory Group member's video diary by visiting: **www.crncc.nihr.ac.uk/researchpeople**

> "Researchers need to make a distinction between child and adult studies. Paediatric studies need to stand alone."

Scottish Children's Research Network – Young Person's Group

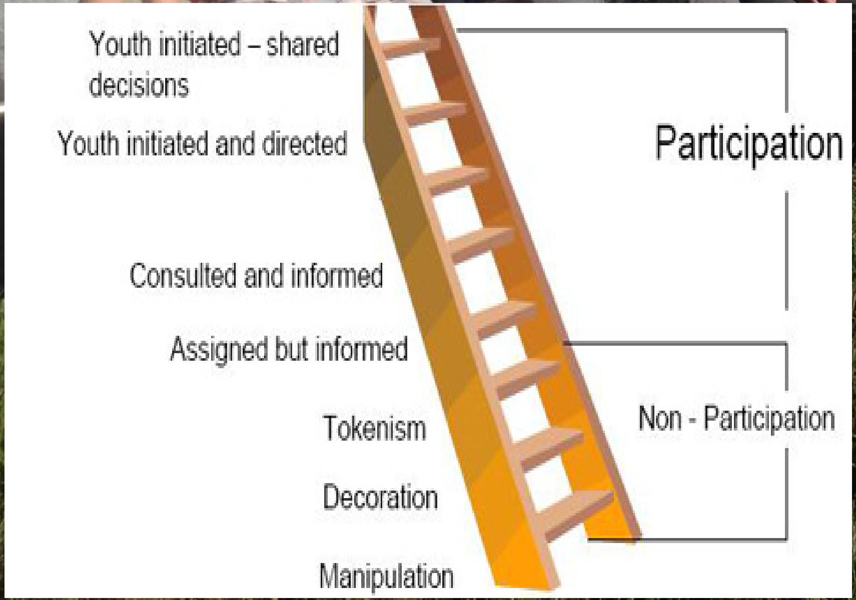
Dr Pamela Dicks
Greg, Joni and Cara YPG members

ScotCRN
Scottish Children's Research Network
Young Persons Group • Grampian

Why a YPG?

Patient Public Involvement - existed

- Lay members on board steering group – professionals
- Parent representatives – prioritisation of areas of research
- Young Persons Advisory Group – young people



Wish list -ScotCRN- Young Persons Group

Advocates for children
Real purpose
Improve clinical trials
Benefit for both Network and the members
Training
Not tokenism
Do able with current staffing

What is KIDS?

Kids and Families Impacting Disease Through Science (KIDS) is an advisory group of children, adolescents and families focused on understanding, communicating and improving medicine, research and innovation for children. KIDS is a collaboration between the American Academy of Pediatrics (AAP) Section on Advances in Therapeutics and Technology (SOATT), local AAP Chapters, children's hospitals, local schools and other partners.

KIDS Speak…

I want to help…

- "I want to help kids have more effective treatments and experiences"
- "We would be doing stuff that really could help, not just talking about it"
- "I have always wanted to play a role in impacting the medical system I just never knew how"
- "I want to help heal kids in the best way possible"
- "I just want to help children with medical conditions and give input on how to make their medical experience better"

I want to learn…

- "I would like to learn more about research in children"
- "I am particularly excited to learn more about unmet pediatric needs"
- "I don't know understand very much about our medical system right now. I want to join to become an educated person and see if I can make a difference"

KIDS Speak…

My opinion matters…

- “I know what it’s like to be a kid”
- “I’ve had pectus carinatum and know what it feels like”
- “Adults will be in need of a child’s perspective for different medical circumstances that involve children”
- “Since I’m a kid, I will be able to relate and connect to other kids better than an adult could”
- “I’m particularly interested in contributing to the design and implementation of clinical studies for children”
- “I would like to voice my opinion and engage in projects that will help children in need of innovation”

KIDS Objectives

Learn, teach, advocate	• for medicine, research and innovation that improves the health and well-being of children
Engage	• in the process through projects and consultation activities with hospitals, researchers, and other partners in the public and private sectors
Provide	• input on research ideas, innovative solutions, unmet pediatric needs and priorities
Contribute	• to the design and implementation of clinical studies for children (e.g., assent, monitoring tools, schedules, etc.)
Serve	• as a critical voice for children and families in the medical, research, and innovation and processes

KIDS Connecticut Pilot

- Launched first KIDS Team in Connecticut in September 2013
- Broad collaboration
  - AAP Section on Advances in Therapeutics and Technology
  - Connecticut Chapter of AAP
  - Connecticut Children's Medical Center
  - Yale-New Haven Children's Hospital
  - Pfizer Inc
  - Local schools (S.T.E.M., other)
- Serve as model for future KIDS Teams

Current Projects

- Write for Life (David Tabatsky)
  - Expansion of project to children
- Sickle Cell Disease
  - Creation of videos on a) role of research in SCD, and b) importance of research and innovation to children
  - Evaluation of proposed website interface for pediatric SCD trial participants
- Global Alliance for Pediatric Therapeutics
  - Participation in pediatric assent project
- MediKidz
  - Input into medical education materials for children
- American Academy of Pediatrics
  - Exploring role for KIDS network in HealthyChildren.org
- Children's Literature
  - Input into materials designed to introduce and explain real life medical illnesses, conditions and circumstances to children

KIDS Collaboration Potential

- Regulators
  - FDA/EMA
- Government agencies
  - NIH/NHLBI/CTSA
  - CDC
- Industry
  - Individual innovator companies (Pfizer, BMS)
  - BIO/PhRMA
- Societies
  - AAP (Committees, Councils, Sections, Chapters, HealthyChildren.Org)
- Pediatric Networks (e.g., GRiP, Enpr-EMA)
- Academia
  - Children's Hospitals, Universities
- Schools
  - STEM (Science, Technology, Engineering, Math)
  - Local public/private schools
- Departments of Public Health
- Innovators

KIDS On The Road…

- ☑ AAP Healthy Children Expo
  - March 8-9, Chicago, IL
  - Exhibit booth (information, survey-based research, research brochure)
  - Learning Zone program (importance of research & innovation)
- ☑ Pediatric Academic Societies
  - May 3-6, Vancouver, BC
  - Exhibit booth (information, survey-based research, research brochure)
  - Joint activities with KidsCan
- ☐ AAP National Conference and Exhibition (October 10-14)
  - October 10-14, San Diego, CA
  - Exhibit booth (information, survey-based research, research brochure)
  - International KIDS Advisory Network (iKAN) strategic planning meeting

KIDS On The Road…

AAP Expo, Chicago

PAS, Vancouver

PAS, Vancouver

AAP Expo, Chicago

PAS, Vancouver

Rome, Italy

AAP Expo, Chicago

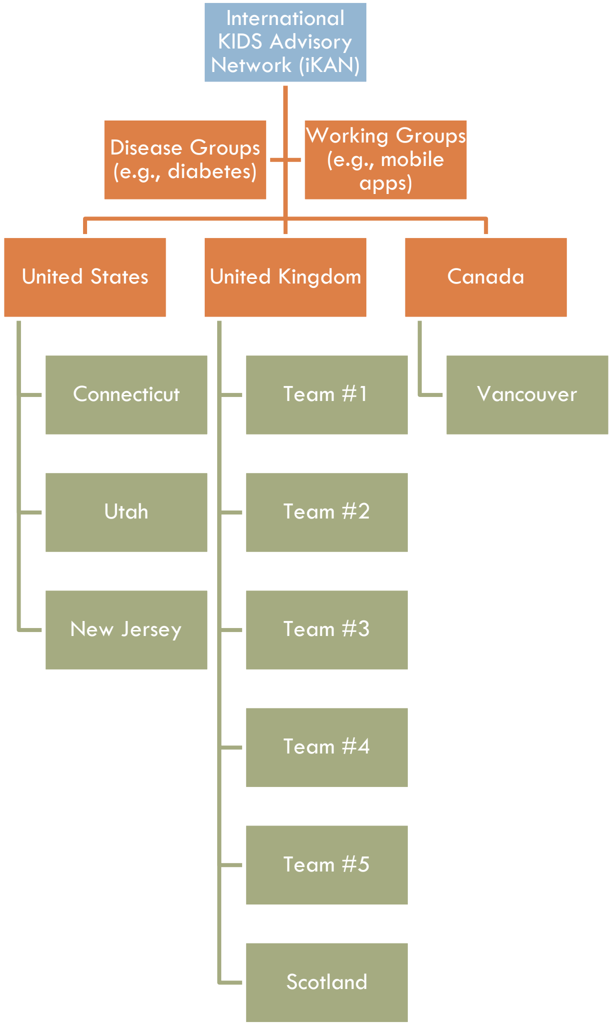
PAS, Vancouver

Pfizer, Connecticut

KIDS Near-Term Opportunities

- Expansion
  - Launch additional KIDS Teams in US and abroad in collaboration with AAP Chapters/pediatric societies, children's hospitals, schools, other partners
- Networking
  - Link KIDS Teams and existing groups into an international KIDS Advisory Network (iKAN)
- Collaboration
  - Explore innovative and impactful collaborative opportunities across states, countries and regions

International Vision



International KIDS Advisory Network (iKAN)

iKAN is an international group of KIDS Teams and young persons advisory groups linked together to provide an opportunity for synergy, communication and collaboration across the growing number of these innovative groups worldwide.

NYU MPH intern (Jessica O'Flaherty) joined team in JUN14

International Vision, continued

iKAN Objectives

Connect	Share	Learn	Collaborate	Advocate
• With similar groups around the world	• Ideas, best practices, challenges, culture	• From one another and capitalize on expertise	• For research, innovation, children and families	• The health and well-being of children around the world

Discussion

- Would you find an international children's advisory network beneficial?
- Are there particular areas of research in which the network would be helpful?
- Would you be interested in starting a local team within this network?
- What elements do you recommend be standardized across the network (e.g., communication, educational materials, etc.)?

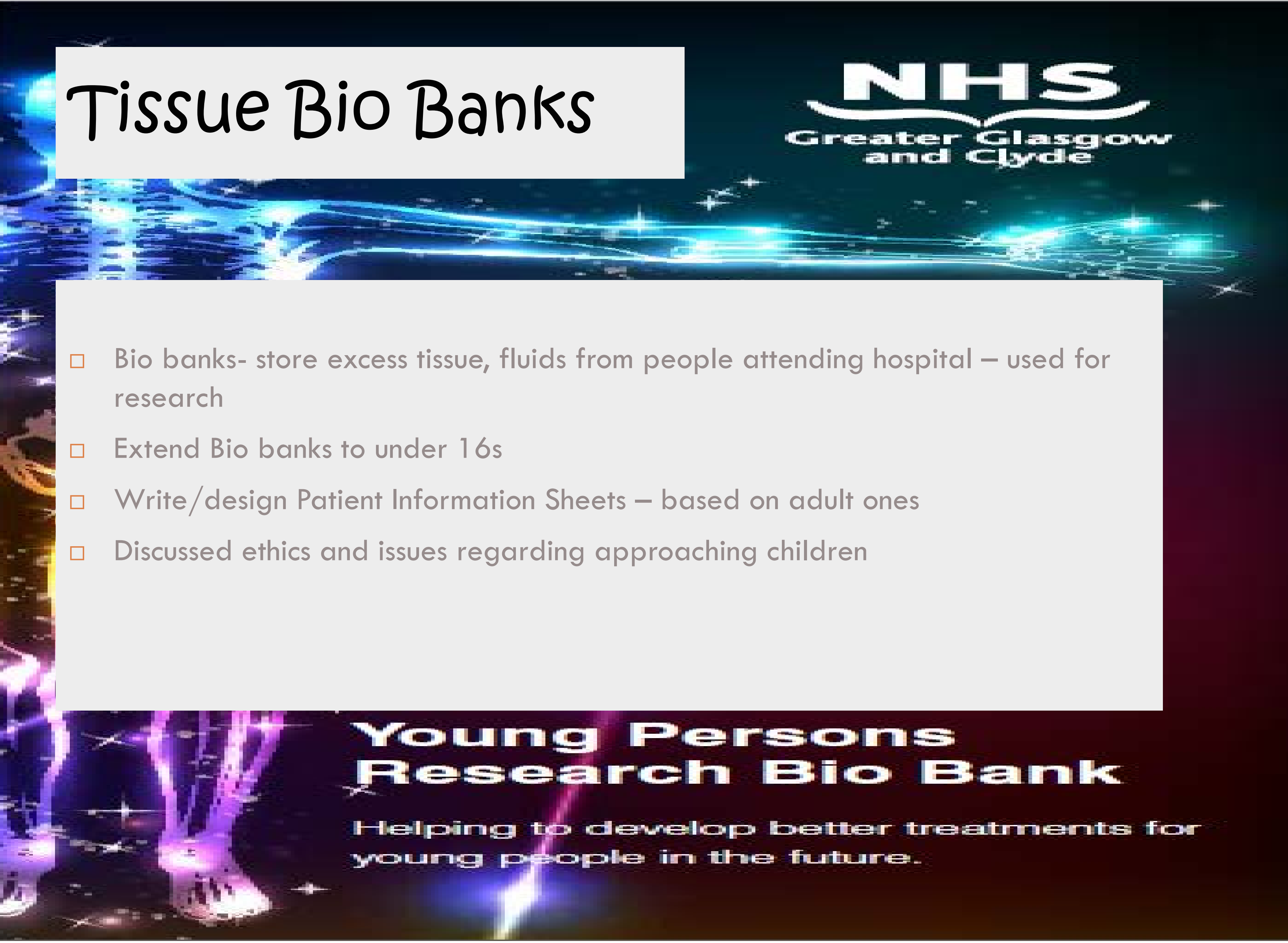
Feedback From Enpr-EMA

- Implement a survey via Survey Monkey to provide us with additional insight about how young persons advisory groups could be of benefit
  - Demographics
  - Would a network of pediatric advisory groups be helpful?
  - Development of local young persons advisory groups
- Link to survey to be sent via email
  - https://www.surveymonkey.com/s/PediatricAdvisoryGroups

Thank You!

Tissue Bio Banks

- Bio banks- store excess tissue, fluids from people attending hospital – used for research
- Extend Bio banks to under 16s
- Write/design Patient Information Sheets – based on adult ones
- Discussed ethics and issues regarding approaching children



NHS
Greater Glasgow
and Clyde

Young Persons
Research Bio Bank

Helping to develop better treatments for young people in the future.

Where we started!

The Use of Surplus Tissue for Medical Research & Education

For children aged 8-12 years

We would like you to gift some of your left over tissues and body fluids for medical research and teaching. This leaflet will help you to decide if this is something you want to do.

Please take time to think it. It's a good idea to talk to your mum or dad about it, before you decide.

What is tissue?

The human body is made up of many cells that are grouped together into tissues. Some tissues form organs such as the lungs, the heart or the appendix. Other tissues are present throughout the body, for example, the bone and skin. Body fluids such as blood, urine and saliva also contain cells and are called tissue.

Doctors or other health care staff may

body fluids than is needed for your care. But, if there is some tissue or body fluid left over that is not required by the doctors looking after you, it could be used for medical research and teaching.

What normally happens to my left over tissue?

The hospital would normally keep some of your tissue as part of your medical record so that it can be examined again if you are ill in the future. However with bigger operations and blood samples most of the tissue left over from testing would be destroyed.

The NHS would like you to gift some of this left over tissue for medical research and teaching. We would also like you to gift any left over tissue from samples that your doctor may have taken in the past.

at the hospital a member of staff will ask you whether or not you wish to take part.

Why is human tissue required for research and teaching?

As well as providing care and treatment, the NHS is responsible for making sure that medical students, nurses and other healthcare professionals get the training they need.

Doctors and scientists also need human tissue for medical research. If samples are collected at different stages of a particular disease it helps to understand how a particular disease or problem starts and develops. They can also try out different drugs and tests on the tissue. They may find new medicines and treatments. They may also find ways of detecting a disease earlier..

Key Changes

NHS
Greater Glasgow
and Clyde

8-12 Year old

- Language was simplified
- Layout -blocked
- Individualised (games, space for name added)
- titive
matio

12-16 Year old

- Language was changed to less patronising
- Layout - blocked
- Graphics added not clip art cartoons
- ive an
ation r

“gift” → “donate”

“nip of” → “sample of”

Examples of graphics we did not like!

The final result!

NHS
Greater Glasgow
and Clyde

Children's Research Tissue Bank
8-12 Years

Helping to develop better treatments for children in the future.

NHS
Greater Glasgow
and Clyde

Young Persons Research Bio Bank

Helping to develop better treatments for young people in the future.