INC
CRITICAL PATH INSTITUTE

International Neonatal Consortium

Second Annual Neonatal Scientific Workshop at the EMA

Welcome

September 12th – 13th, 2016

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Guido Rasi

September 12th – 13th, 2016

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Agenda – September 12th, Morning

9:00 a.m.	**Welcome** GUIDO RASI (EMA)
9:15 a.m.	**Keynote** *Where We Need to Move Neonatology* NEENA MODI (IMPERIAL COLLEGE LONDON)
9:45 a.m.	**Session I:** *Embracing a Research Culture* GERRI BAER (FDA) & MARY SHORT (LILLY), CO-CHAIRS
	Fostering a Culture of Research to Improve Outcomes KELLY WADE (CHILDREN'S HOSPITAL OF PHILADELPHIA)
10:15 – 10:45 a.m.	COFFEE BREAK
10:45 a.m.	**Session I Panel:** *Fostering a Culture of Research: Beliefs, Strengths/Barriers, Needs, INC role/opportunity for Establishing a Research Culture*
12:00 - 1:00 p.m.	LUNCH

Keynote

Where We Need to Move Neonatology

NEENA MODI (IMPERIAL COLLEGE LONDON)

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Where We Need to Move Neonatology

Neena Modi

Professor of Neonatal Medicine, Imperial College London

President, UK Royal College of Paediatrics and Child Health

@RCPCHPresident

CRITICAL PATH INSTITUTE

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

For most of human history infants and children were considered possessions, at best passive objects of care and charity

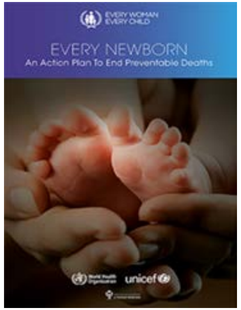
The Declaration of the Rights of the Child was not until 1959 followed 30 years later in 1989 by the Convention on the Rights of the Child

The Convention on the Rights of the Child changed the way children are viewed, requiring them to be treated as individuals with a distinct set of rights, and enshrining this in law

A SUMMARY OF THE UN CONVENTION ON THE RIGHTS OF THE CHILD

unicef

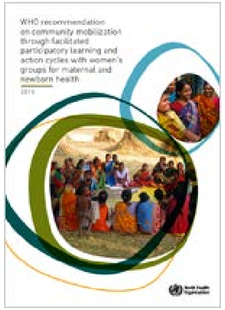
Millennium Development Goals


EVERY WOMAN EVERY CHILD

EVERY NEWBORN

An Action Plan To End Preventable Deaths

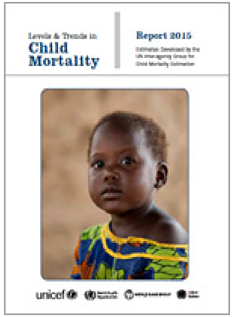
unicef


WHO recommendation on community mobilization through facilitated participatory learning and action cycles with women's groups for maternal and newborn health

2015


Born Too Soon

The Global Action Report on Preterm Birth


Levels & Trends in Child Mortality

Report 2015

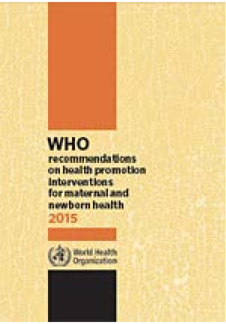
unicef


WHO/UNICEF JOINT STATEMENT

Home visits for the newborn child: a strategy to improve survival

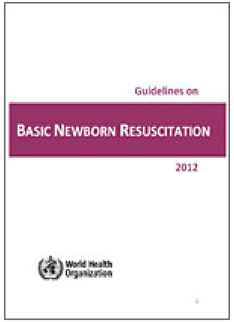
World Health Organization

unicef


WHO recommendations on health promotion interventions for maternal and newborn health

2015

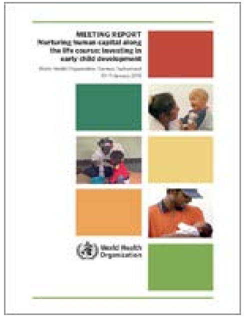
World Health Organization


Guidelines on

BASIC NEWBORN RESUSCITATION

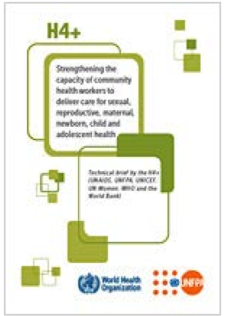
2012

World Health Organization


MEETING REPORT

Nurturing human capital along the life course: Investing in early child development

World Health Organization

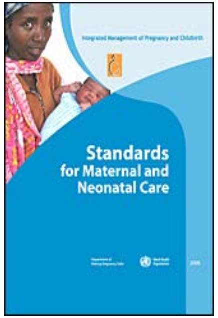

H4+

Strengthening the capacity of community health workers to deliver care for sexual, reproductive, maternal, newborn, child and adolescent health

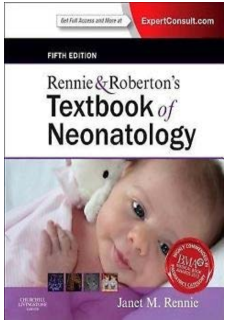
Technical brief by the H4+ (UNAIDS, UNFPA, UNICEF, UN Women, WHO and the World Bank)

World Health Organization

UNFPA


Integrated Management of Pregnancy and Childbirth

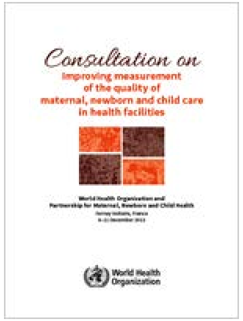
Standards for Maternal and Neonatal Care


Get Full Access and More at ExpertConsult.com

FIFTH EDITION

Rennie & Roberton's Textbook of Neonatology

Janet M. Rennie


Consultation on

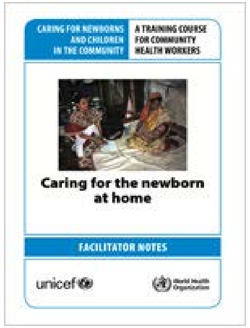
Improving measurement of the quality of maternal, newborn and child care in health facilities

World Health Organization


GUIDELINE

Managing possible serious bacterial infection in young infants when referral is not feasible

World Health Organization


CARING FOR NEWBORNS AND CHILDREN IN THE COMMUNITY

A TRAINING COURSE FOR COMMUNITY HEALTH WORKERS

Caring for the newborn at home

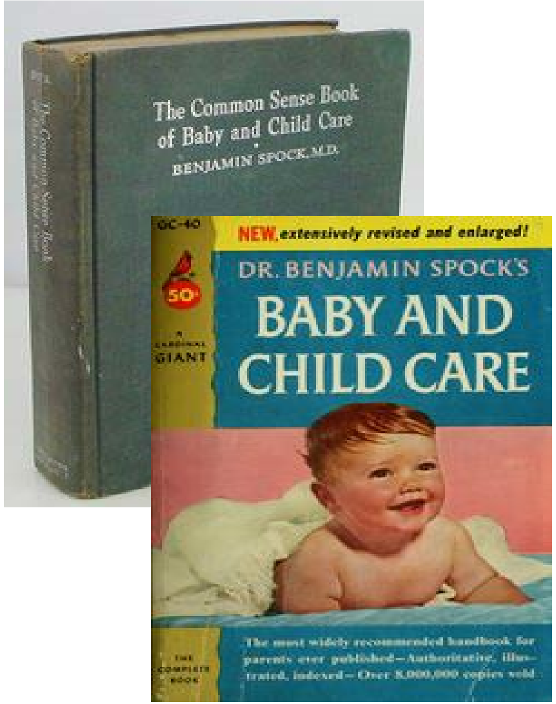
FACILITATOR NOTES

unicef

World Health Organization

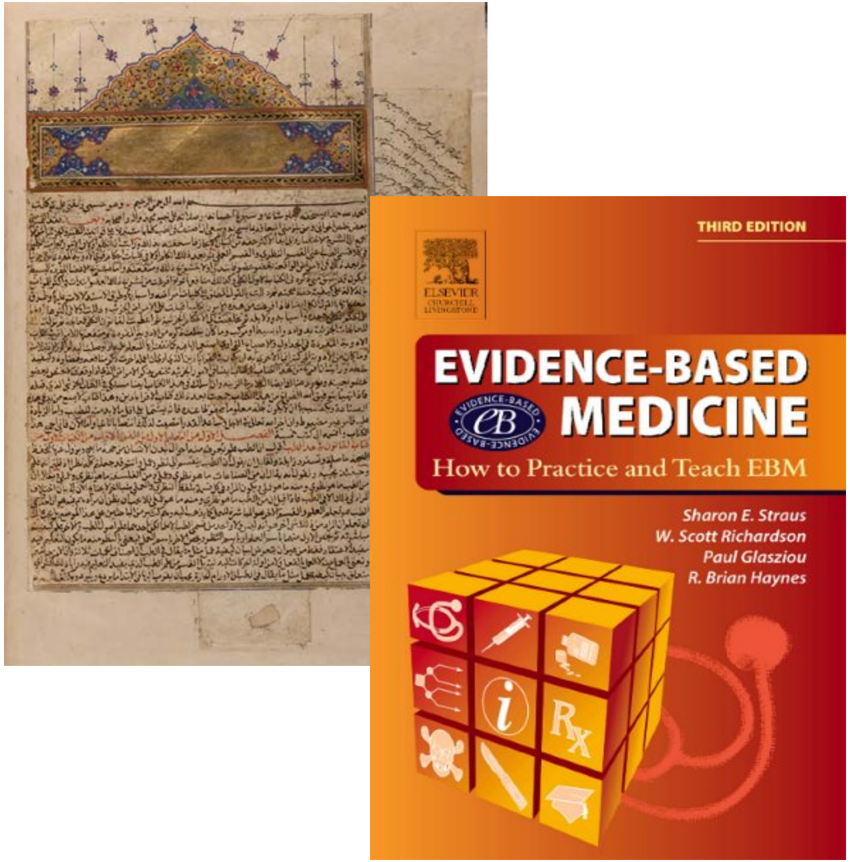
Much of what is taught today may be wrong

- Prone sleeping for newborn babies: increased Sudden Infant Death
- Routine separation of mothers and babies: decreased breast-feeding
- Postnatal steroids for chronic lung disease in preterm babies: 3-fold increase in cerebral palsy
- Resuscitation of newborn babies in 100% oxygen compared with air: 3-fold increase in mortality



For 50 years a best seller second only to the Bible

Evidence based medicine



THIRD EDITION

ELSEVIER CHURCHILL LIVINGSTONE

EVIDENCE-BASED MEDICINE

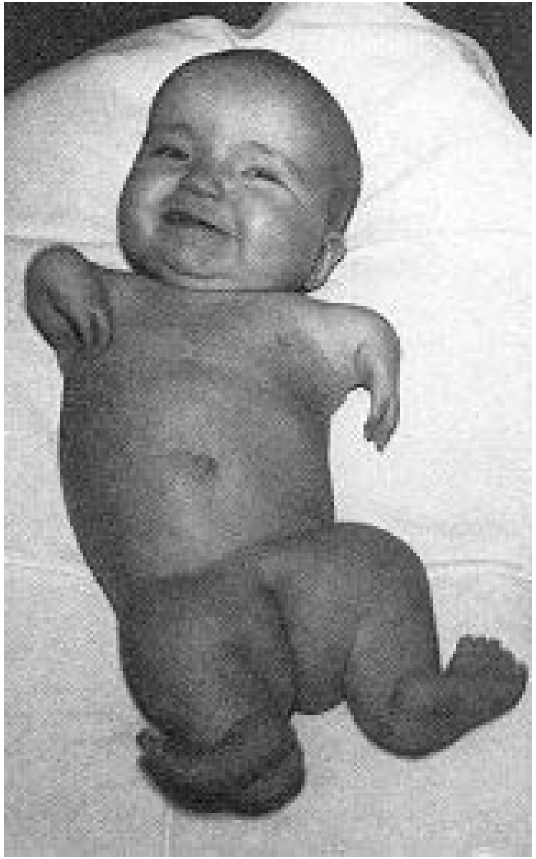
How to Practice and Teach EBM

Sharon E. Straus
W. Scott Richardson
Paul Glasziou
R. Brian Haynes

- Archie Cochrane, modern founder of "evidence-based" medicine
- His book Effectiveness and Efficiency (1972) led to increasing acceptance of controlled studies during the 70s and 80s paving the way for EBM, a term coined by Guyatt in 1990
- Ibn Sina (Avicenna) (AD 981-1037) an eminent figure in Islamic learning
- His Canon of Medicine contained a precise set of rules for testing medications
- Widely held to be the father of evidence-based medicine

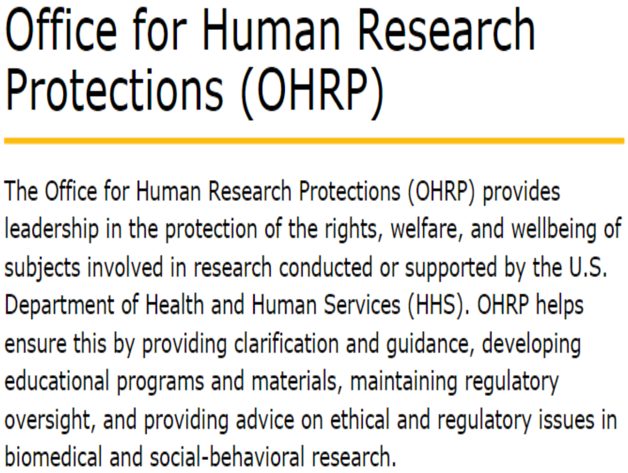
Testing children’s medicines in children

- Dr Louis Lasagna, prominent advocate in the 1950s for randomised, placebo controlled trials, drug licensing to be underpinned by evidence, and the necessity of informed consent for medical research
- Conducted the first placebo controlled trial of a medication prior to market launch
- Trial undertaken by Dr Lasagna; showed the new medication to be “effective and safe” as a sedative and anti-emetic
- By mid-1950s marketed globally



The SUPPORT trial: a breakdown between the public and researchers

A comparative effectiveness trial, comparing the use of oxygen in practices in widespread every-day use



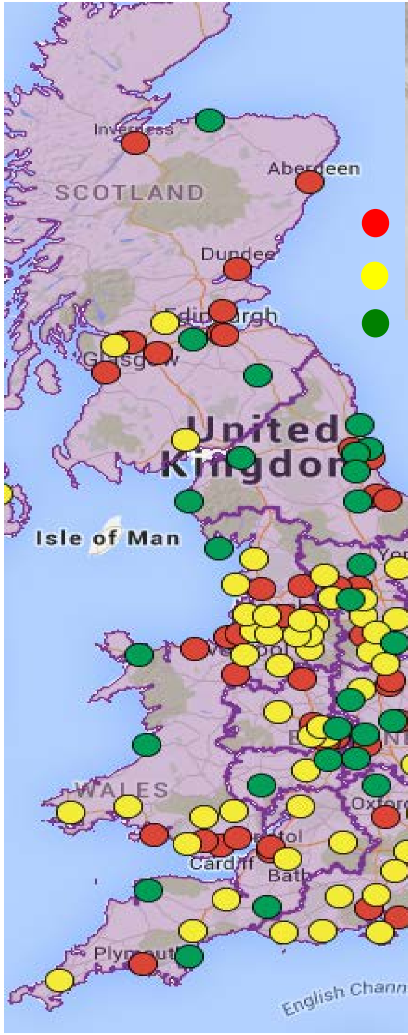
Office for Human Research Protections (OHRP)

The Office for Human Research Protections (OHRP) provides leadership in the protection of the rights, welfare, and wellbeing of subjects involved in research conducted or supported by the U.S. Department of Health and Human Services (HHS). OHRP helps ensure this by providing clarification and guidance, developing educational programs and materials, maintaining regulatory oversight, and providing advice on ethical and regulatory issues in biomedical and social-behavioral research.

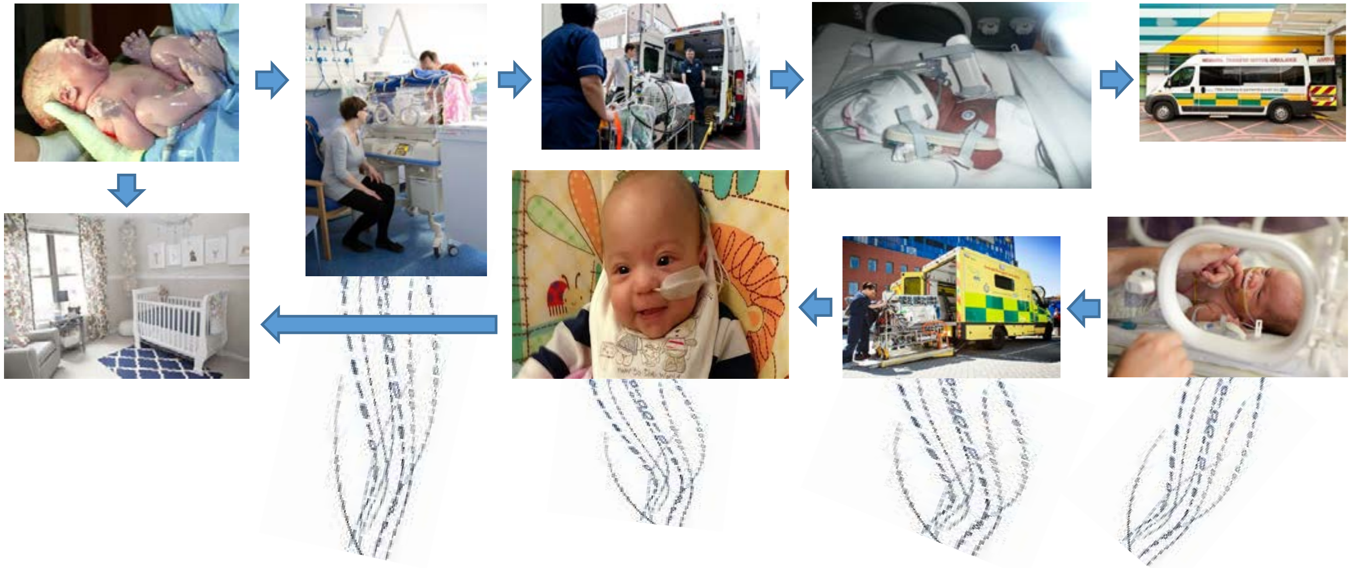
- *“The experimental study exposed 1,316 premature infants to increased risk of blindness, brain injury and death without informing parents of the risks to their babies or the true nature and purpose of the research”*
- *"This is an object and unacceptable failure to protect human subjects in clinical trials and is undoubtedly because of political pressure“*
- *Informed consent "must not be compromised simply to satisfy the desire of medical researchers for expediency in their quest to advance medical knowledge"*

A new paradigm to advance neonatology

Reducing uncertainties, evaluating care, and testing treatments, more quickly and efficiently, at less cost, and placing less burden upon clinical teams and families

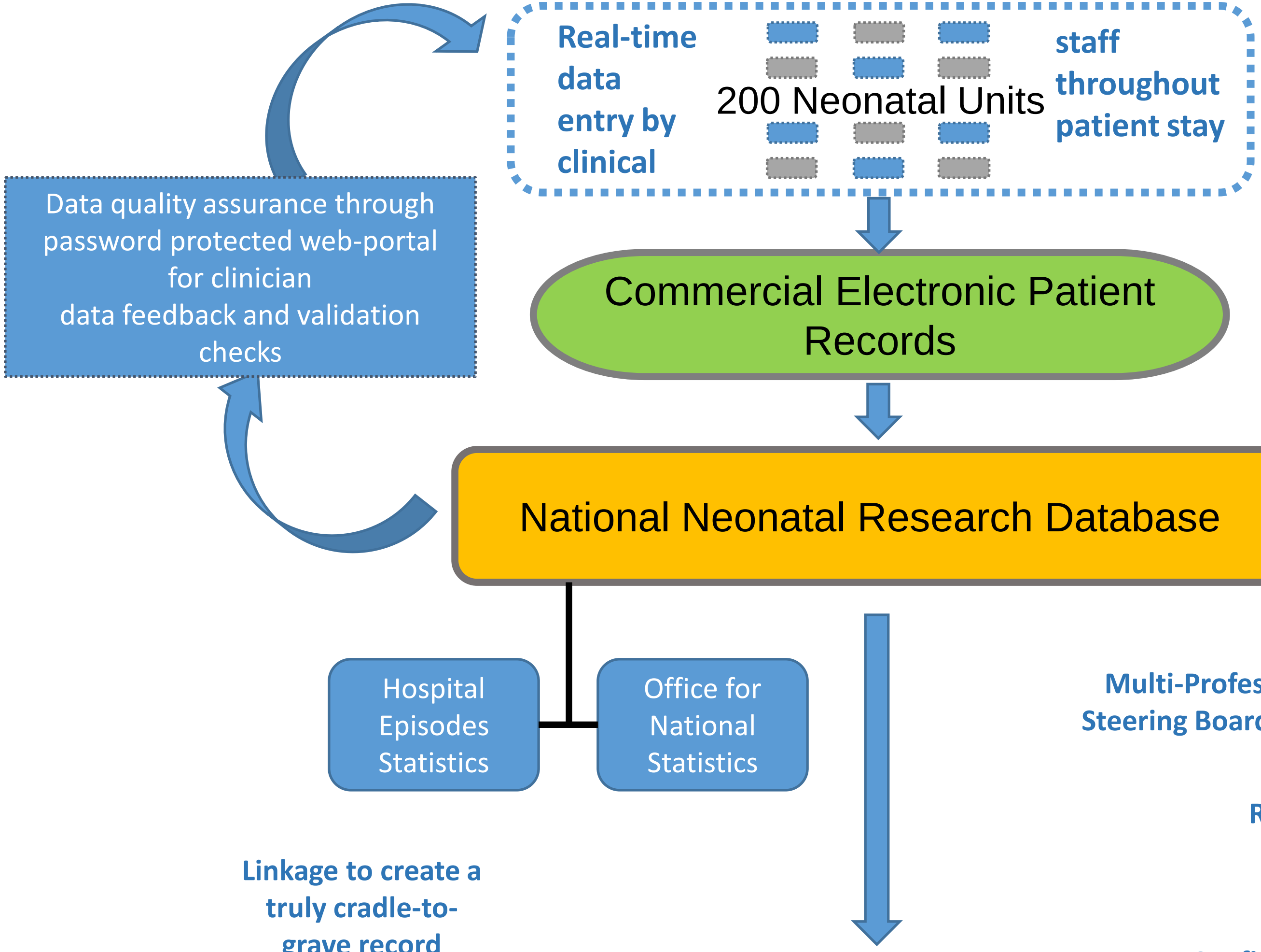


Special Care Baby Unit



Commercial Electronic Patient Record
200 Neonatal Units

National Neonatal Research Database
Neonatal Data Analysis Unit
Imperial College London and
Chelsea and Westminster NHS Foundation Trust



Real-time data entry by clinical staff throughout patient stay

200 Neonatal Units

Commercial Electronic Patient Records

National Neonatal Research Database

Data quality assurance through password protected web-portal for clinician data feedback and validation checks

Hospital Episodes Statistics

Office for National Statistics

Linkage to create a truly cradle-to-grave record

Multiple Outputs

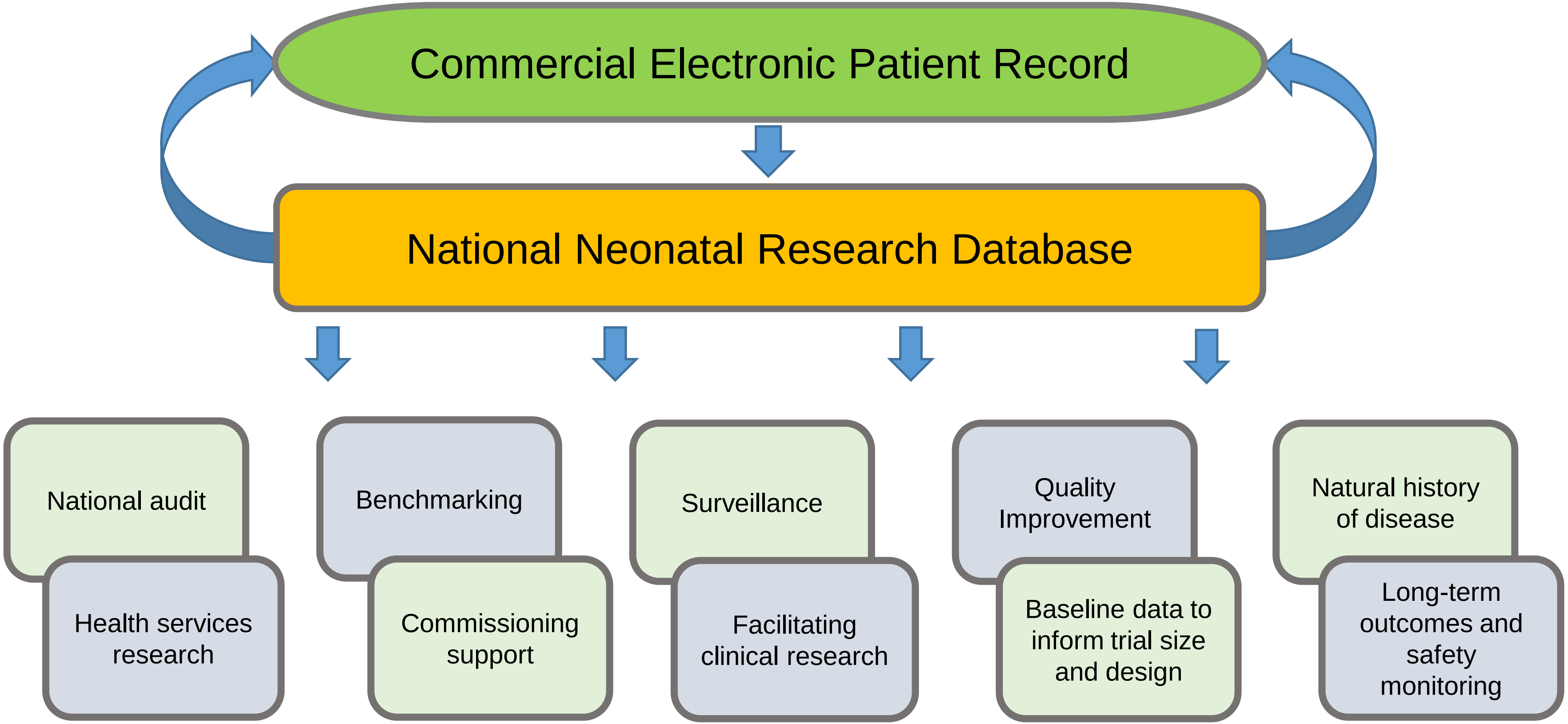
UK Neonatal Collaborative

Creation of new NHS Information Standard (Neonatal Data Set)

Extraction of the Neonatal Data Set from Electronic Patient Records to create the NNRD, saving £600K pa

Multi-Professional Neonatal Data Analysis Unit Steering Board with parents involved at every step

Regulatory approvals:
Caldicott Guardians
Lead Clinicians
Research Ethics
Confidentiality Advisory Group



Commercial Electronic Patient Record

National Neonatal Research Database

National audit

Health services research

Benchmarking

Commissioning support

Surveillance

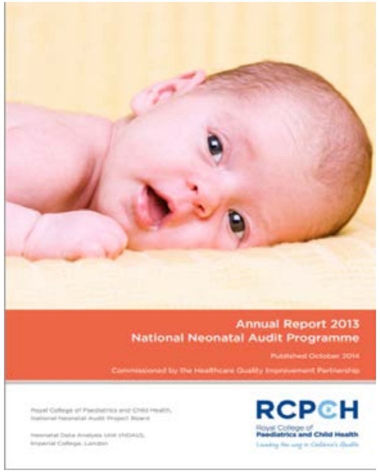
Facilitating clinical research

Quality Improvement

Baseline data to inform trial size and design

Natural history of disease

Long-term outcomes and safety monitoring

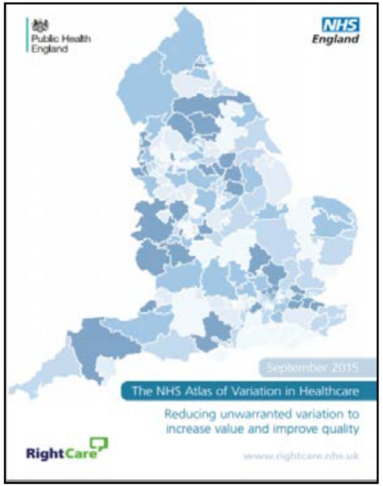


Annual Report 2013
National Neonatal Audit Programme
Published October 2014
Commissioned by the Healthcare Quality Improvement Partnership

Royal College of Paediatrics and Child Health,
National Neonatal Audit Project Board

Neonatal Data Analysis Unit (NDAU),
Imperial College, London

RCPCH
Royal College of
Paediatrics and Child Health
Leading the way in Children's Health



Public Health England

NHS England

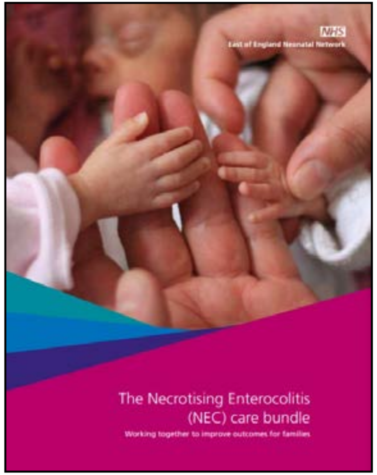
September 2015

The NHS Atlas of Variation in Healthcare

Reducing unwarranted variation to increase value and improve quality

RightCare

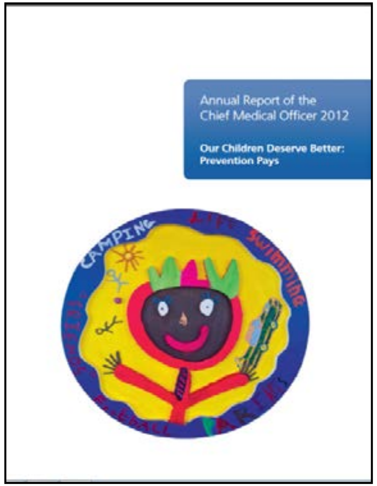
www.rightcare.nhs.uk



NHS
East of England Neonatal Network

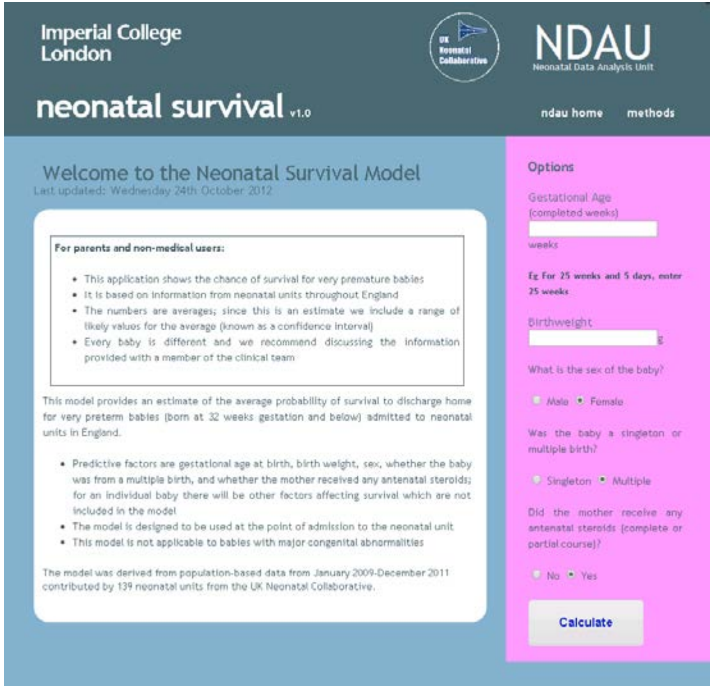
The Necrotising Enterocolitis (NEC) care bundle

Working together to improve outcomes for families



Annual Report of the Chief Medical Officer 2012

Our Children Deserve Better: Prevention Pays



Imperial College London

UK Neonatal Collaborative

NDAU
Neonatal Data Analysis Unit

neonatal survival v1.0

Welcome to the Neonatal Survival Model

Last updated: Wednesday 24th October 2012

For parents and non-medical users:

- This application shows the chance of survival for very premature babies
- It is based on information from neonatal units throughout England
- The numbers are averages; since this is an estimate we include a range of likely values for the average (known as a confidence interval)
- Every baby is different and we recommend discussing the information provided with a member of the clinical team

This model provides an estimate of the average probability of survival to discharge home for very preterm babies (born at 32 weeks gestation and below) admitted to neonatal units in England.

- Predictive factors are gestational age at birth, birth weight, sex, whether the baby was from a multiple birth, and whether the mother received any antenatal steroids; for an individual baby there will be other factors affecting survival which are not included in the model
- The model is designed to be used at the point of admission to the neonatal unit
- This model is not applicable to babies with major congenital abnormalities

The model was derived from population-based data from January 2009-December 2011 contributed by 139 neonatal units from the UK Neonatal Collaborative.

Options

Gestational Age (completed weeks)
weeks

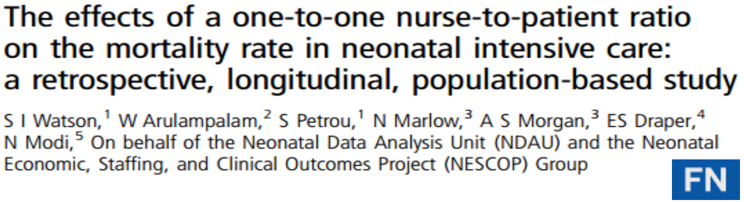
Eg For 25 weeks and 5 days, enter 25 weeks

Birthweight
g

What is the sex of the baby?
○ Male ● Female

Was the baby a singleton or multiple birth?
○ Singleton ● Multiple

Did the mother receive any antenatal steroids (complete or partial course)?
○ No ● Yes



The effects of a one-to-one nurse-to-patient ratio on the mortality rate in neonatal intensive care: a retrospective, longitudinal, population-based study

S I Watson,[1] W Arulampalam,[2] S Petrou,[1] N Marlow,[3] A S Morgan,[3] ES Draper,[4] N Modi,[5] On behalf of the Neonatal Data Analysis Unit (NDAU) and the Neonatal Economic, Staffing, and Clinical Outcomes Project (NESCOP) Group

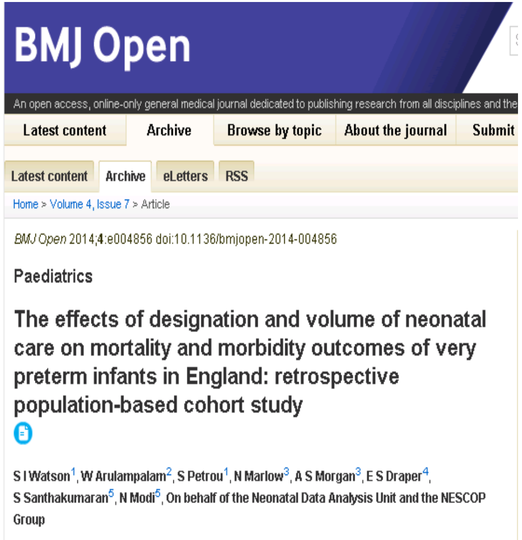
Arch Dis Child Fetal Neonatal Ed published online February 9, 2016

Impact of managed clinical networks on NHS specialist neonatal services in England: population based study

BMJ 2012;344:e2105

C Gale *clinical research fellow*, S Santhakumaran *statistician*, S Nagarajan *data analyst*, Y Statnikov *data analyst*, N Modi *professor of neonatal medicine*, on behalf of the Neonatal Data Analysis Unit and the Medicines for Neonates Investigator Group

Section of Neonatal Medicine, Department of Medicine, Chelsea and Westminster Campus, Imperial College London, London SW10 9NH, UK



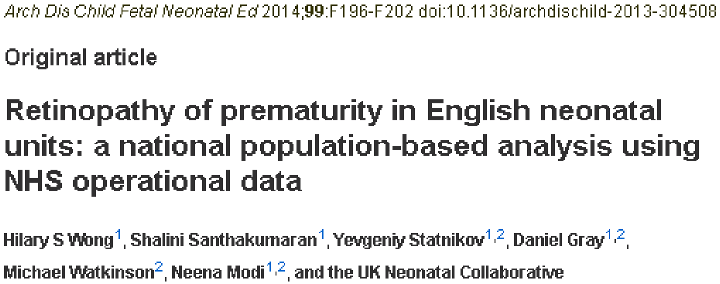
An open access, online-only general medical journal dedicated to publishing research from all disciplines and the

BMJ Open 2014;**4**:e004856 doi:10.1136/bmjopen-2014-004856

Paediatrics

The effects of designation and volume of neonatal care on mortality and morbidity outcomes of very preterm infants in England: retrospective population-based cohort study

S I Watson[1], W Arulampalam[2], S Petrou[1], N Marlow[3], A S Morgan[3], E S Draper[4], S Santhakumaran[5], N Modi[5], On behalf of the Neonatal Data Analysis Unit and the NESCOP Group



Arch Dis Child Fetal Neonatal Ed 2014;**99**:F196-F202 doi:10.1136/archdischild-2013-304508

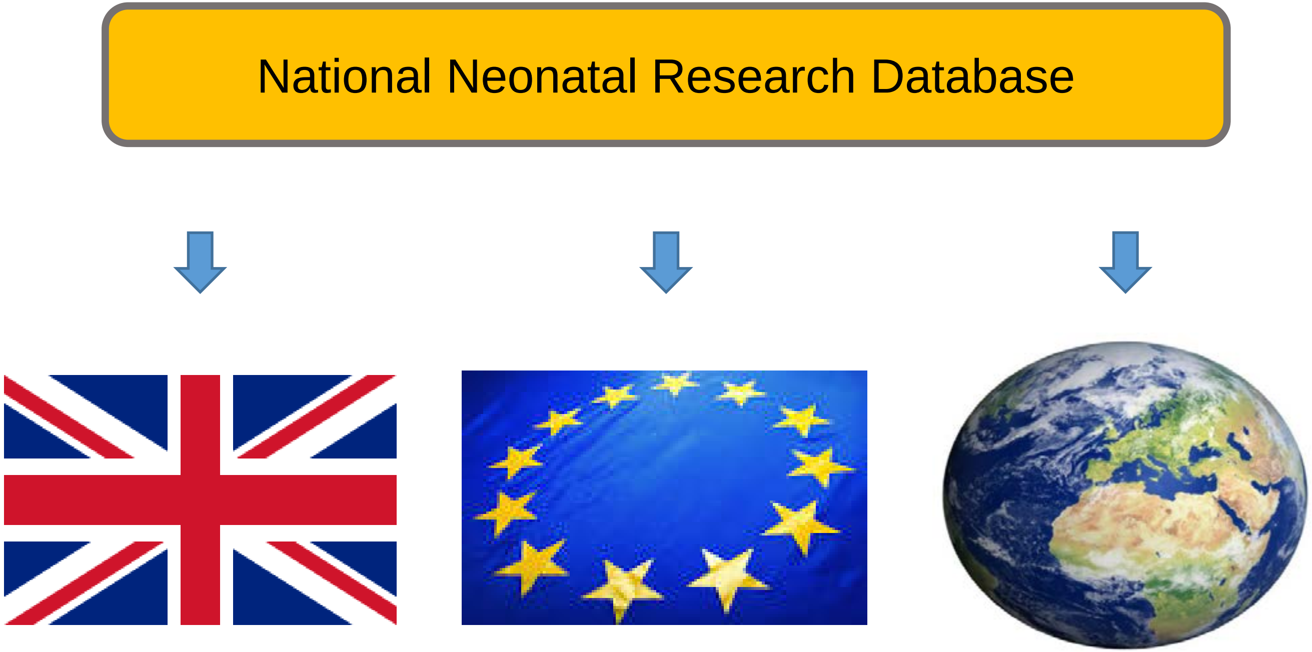
Original article

Retinopathy of prematurity in English neonatal units: a national population-based analysis using NHS operational data

Hilary S Wong[1], Shalini Santhakumaran[1], Yevgeniy Statnikov[1,2], Daniel Gray[1,2], Michael Watkinson[2], Neena Modi[1,2], and the UK Neonatal Collaborative

Neonatal randomised point-of-care trials are feasible and acceptable in the UK: results from two national surveys

Christopher Gale, **Neena Modi** on behalf of the WHEAT trial development group



National Neonatal Research Database

Working in partnership

Scientists and society

- James Lind Library (www.jameslindlibrary.org)
- Testing Treatments Interactive (ww.testingtreatments.org)
- INVOLVE (www.invo.org.uk)
- Science Media Centre (www.sciencemediacentre.org)
- Sense about Science (www.senseaboutscience.org)
- *& Us*: the RCPCH platform for children, young people, parents, carers and families to join us in improving child health (www.rcpch.ac.uk)

Research & Us

Infants', Children's and Young People's Child Health Research Charter

& Us
The voice of children, young people and families

Remember it's about me; involve and support me at every stage and keep me safe from harm.

Empower me, my family, and the people caring for me; help us to understand and feel more confident about research.

Support me, my family, and the people caring for me; give us time to understand research processes, how to assess risks and benefits, and what it will mean to be involved.

Engage with me and my family; listen to our questions and ideas, so we can help you to help us benefit from research.

Actively gain my consent or assent and explain my right to change my mind and withdraw at any time.

Rights are important to me; my right to understand research, be involved in decisions, be respected, and to help others benefit from research.

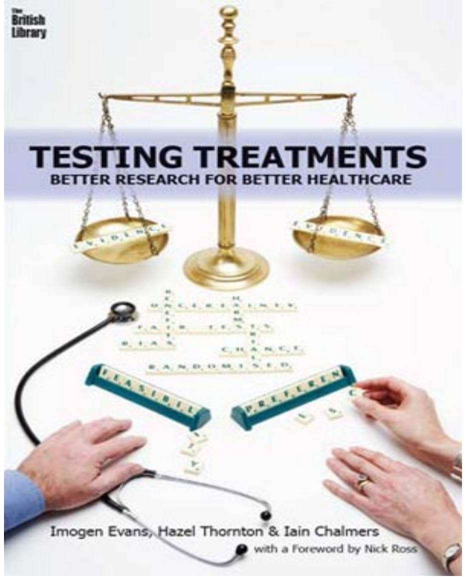
Communicate with me directly and clearly; make it easy for me to talk to someone about the research when I have questions or ideas.

Help others by sharing our stories; the things that worked and the things that didn't.

Contact your local engagement lead for information:

Go to www.rcpch.ac.uk/cyp-research-charter for more information about external resources and useful links to support child health research.

RCPCH
Royal College of Paediatrics and Child Health
Leading the way in Children's Health



The British Library

TESTING TREATMENTS

BETTER RESEARCH FOR BETTER HEALTHCARE

Imogen Evans, Hazel Thornton & Iain Chalmers
with a Foreword by Nick Ross

Scientists and funders

The UK Child Health Research Collaboration is a growing partnership between charities, other funders of child health research and other interested organisations, that aims to improve child health through increasing and strengthening research

The UK Child Health Research Collaboration is hosted by the Royal College of Paediatrics and Child Health, and has the support of the Medical Research Council, Wellcome Trust, Cancer Research UK, and National Institute for Health Research

UKCHRC

UK Child Health Research Collaboration

Informing, educating, training

Training clinicians to evaluate and advance care

The UK Royal College of Paediatrics and Child Health introduced a National Neonatal Audit Programme in 2009, generic research skills training into the curriculum in 2014, and established a Research Capacity Development Fund in 2016

Creating a national child health research culture

Advocate

A healthy nation: strengthening child health research in the UK

Neena Modi, Howard Clark, Ingrid Wolfe, Anthony Costello, Helen Budge, for the writing group of the Royal College of Paediatrics and Child Health Commission on Child Health Research

Despite a general acknowledgment that research in children is necessary and ethical, the evidence base for child-specific treatments is still sparse. We investigated children's biomedical and health services research in the UK in relation to training, infrastructure and activity, research evidence, and visibility. We show that excellent opportunities for career researchers exist through a competitive, national integrated academic training programme, but that the number of academic paediatricians has decreased by 18% between 2000 and 2011, falling from 11·3% to 5·9% of the

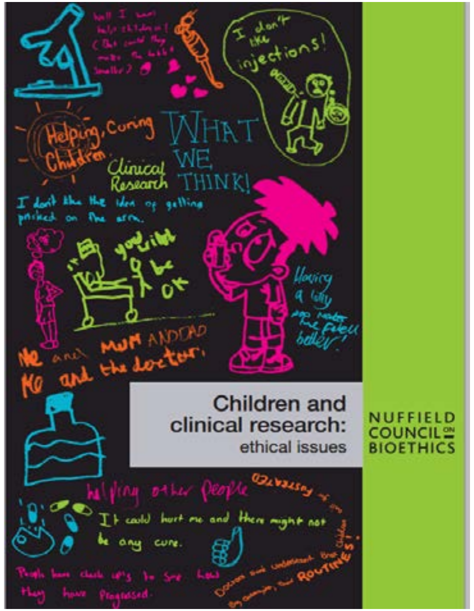
Lancet 2013; 381: 73–87

Published Online
November 20, 2012
http://dx.doi.org/10.1016/S0140-6736(12)61818-2

Turning the Tide:
Harnessing the power of child health research

A report by the Royal College of Paediatrics & Child Health Commission on Child Health Research

RCPCH
Royal College of Paediatrics and Child Health



Children and clinical research: ethical issues

NUFFIELD COUNCIL ON BIOETHICS

THE LANCET

"The time has come to protect children and young people *through* research not *from* research."

Challenging convention

Challenge convention: research ethics evolve

The British Paediatric Association, the forerunner of the Royal College of Paediatrics and Child Health first published guidance on research in 1980 challenging the then accepted view by stating “*research involving children is important*”, “*should be supported and encouraged*” and “*research which involves a child and is of no benefit to that child (non-therapeutic research) is not necessarily either unethical or illegal*”

Updated guidance issued in 2000 and 2014

Archives of
Disease in Childhood

An international peer-reviewed journal for health professionals and researchers covering conception to adolesce

Arch Dis Child 2014;99:887-891 doi:10.1136/archdischild-2014-306444

Leading article

Guidance on clinical research involving infants, children and young people: an update for researchers and research ethics committees

Neena Modi[1], Jyotsna Vohra[1], Jennifer Preston[2], Catherine Elliott[3], William Van't Hoff[2], Jane Coad[4], Faith Gibson[4], Linda Partridge[5], Joe Brierley[1], Vic Larcher[1], Anne Greenough[1,6] for a Working Party of the Royal College of Paediatrics and Child Health

Correspondence to

Professor Neena Modi, Royal College of Paediatrics and Child Health, 5-11 Theobald's Road, London WC1X 8SH, UK; n.modi@imperial.ac.uk

Clinical uncertainty is a patient safety issue

- Where the evidence base is uncertain the patient is better served by receiving treatment unaffected by clinician bias, and care delivered along a clearly designed, closely monitored pathway, i.e. a clinical trial
- Propose integration of research into clinical care
- Key differences between comparative effectiveness trials and research involving new treatments are randomization as standard-of-care, opt-out the default rather than opt-in, and explicit mention of the possibility of inclusion-benefit
- Peer review, regulatory approval, explanation, information and ability to refuse participation, the same as for experimental medicine research
- Fulfils the four cardinal principles of research

- **Justice:** the patient is treated fairly and fully informed
- **Beneficence:** the doctor fulfils his/her obligation to act in the patient's best interests
- **Non-maleficence:** the doctor fulfils his/her obligation to do no harm
- **Autonomy:** the parent decides, freely and without coercion

Improving the efficiency of neonatal research

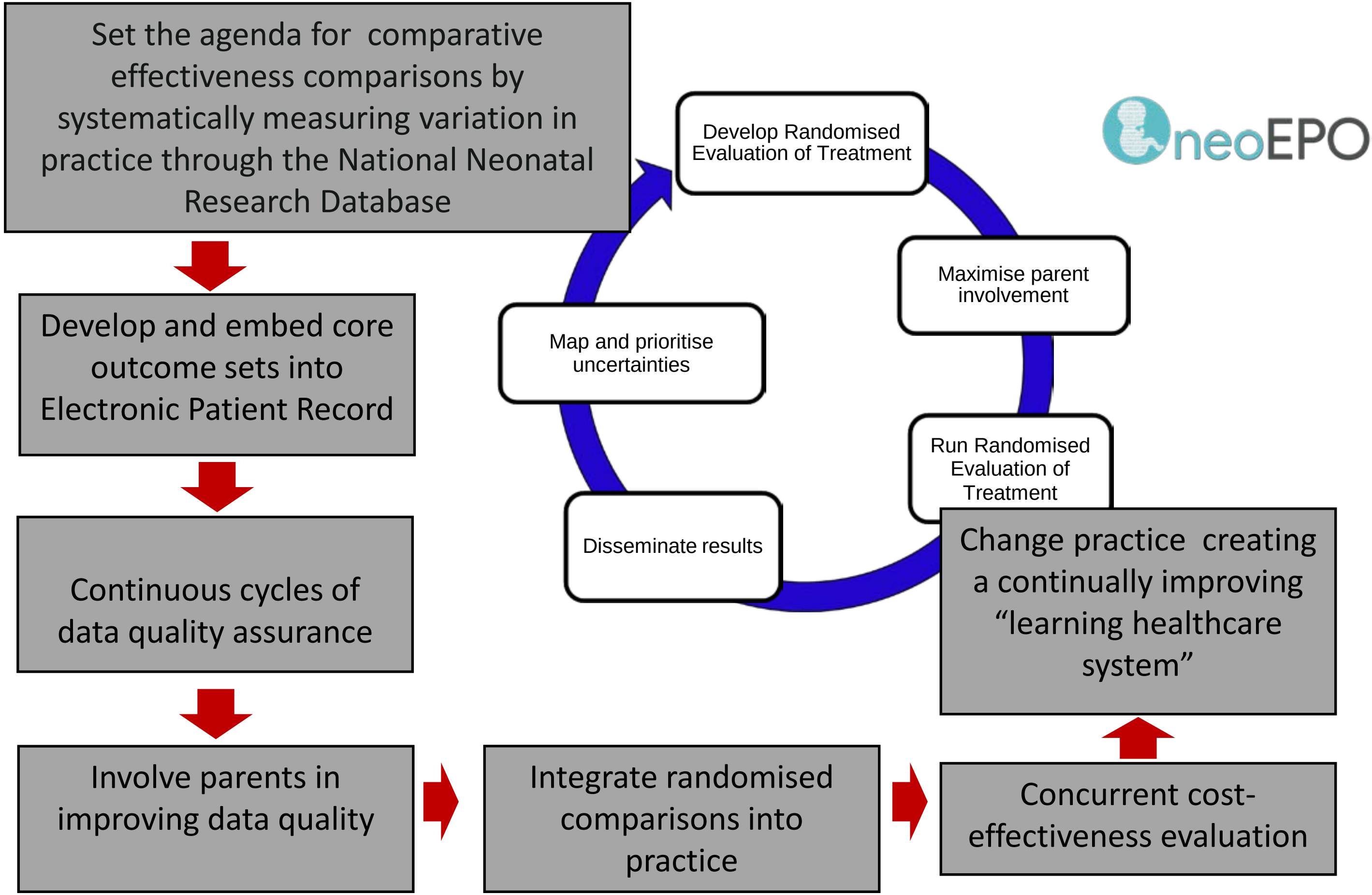
(Hyde, Gale, Modi, In Press)

Neonatal comparative-effectiveness, randomised controlled trial developed with parents

Incorporated four approaches to improve efficiency of comparative effective research:

- Point-of-care design using Electronic Patient Records for patient identification, randomisation and data acquisition
- Short two-page information sheet
- Explicit mention of possibility of inclusion benefit
- Opt-out consent with enrollment as the default

neoEPOCH



Set the agenda for comparative effectiveness comparisons by systematically measuring variation in practice through the National Neonatal Research Database

Develop and embed core outcome sets into Electronic Patient Record

Continuous cycles of data quality assurance

Involve parents in improving data quality

Integrate randomised comparisons into practice

Concurrent cost-effectiveness evaluation

Change practice creating a continually improving "learning healthcare system"

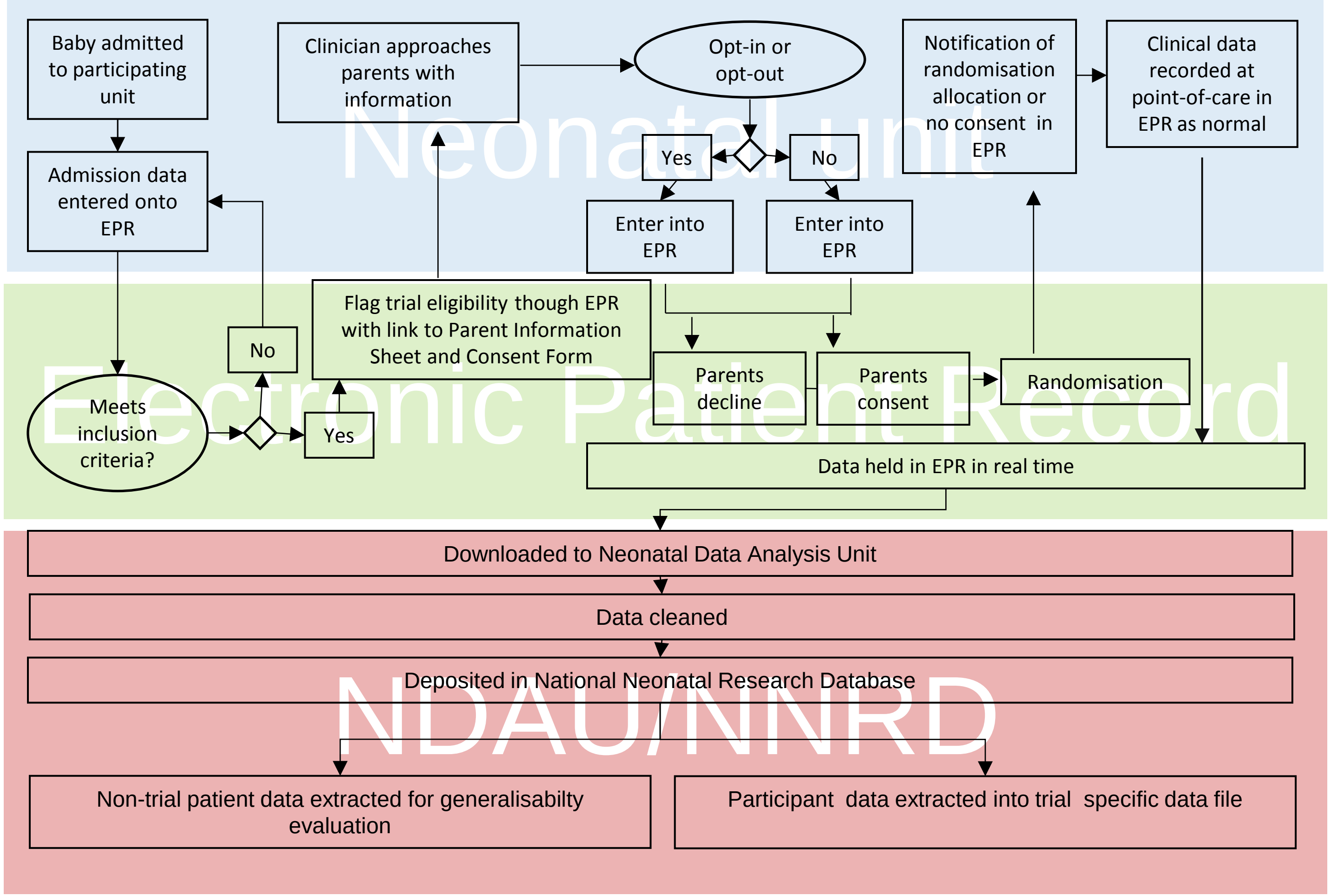
Develop Randomised Evaluation of Treatment

Maximise parent involvement

Run Randomised Evaluation of Treatment

Disseminate results

Map and prioritise uncertainties



Neonatal unit

Baby admitted to participating unit

Admission data entered onto EPR

Clinician approaches parents with information

Opt-in or opt-out

Yes

No

Enter into EPR

Enter into EPR

Notification of randomisation allocation or no consent in EPR

Clinical data recorded at point-of-care in EPR as normal

Electronic Patient Record

Meets inclusion criteria?

No

Yes

Flag trial eligibility though EPR with link to Parent Information Sheet and Consent Form

Parents decline

Parents consent

Randomisation

Data held in EPR in real time

NDAU/NNRD

Downloaded to Neonatal Data Analysis Unit

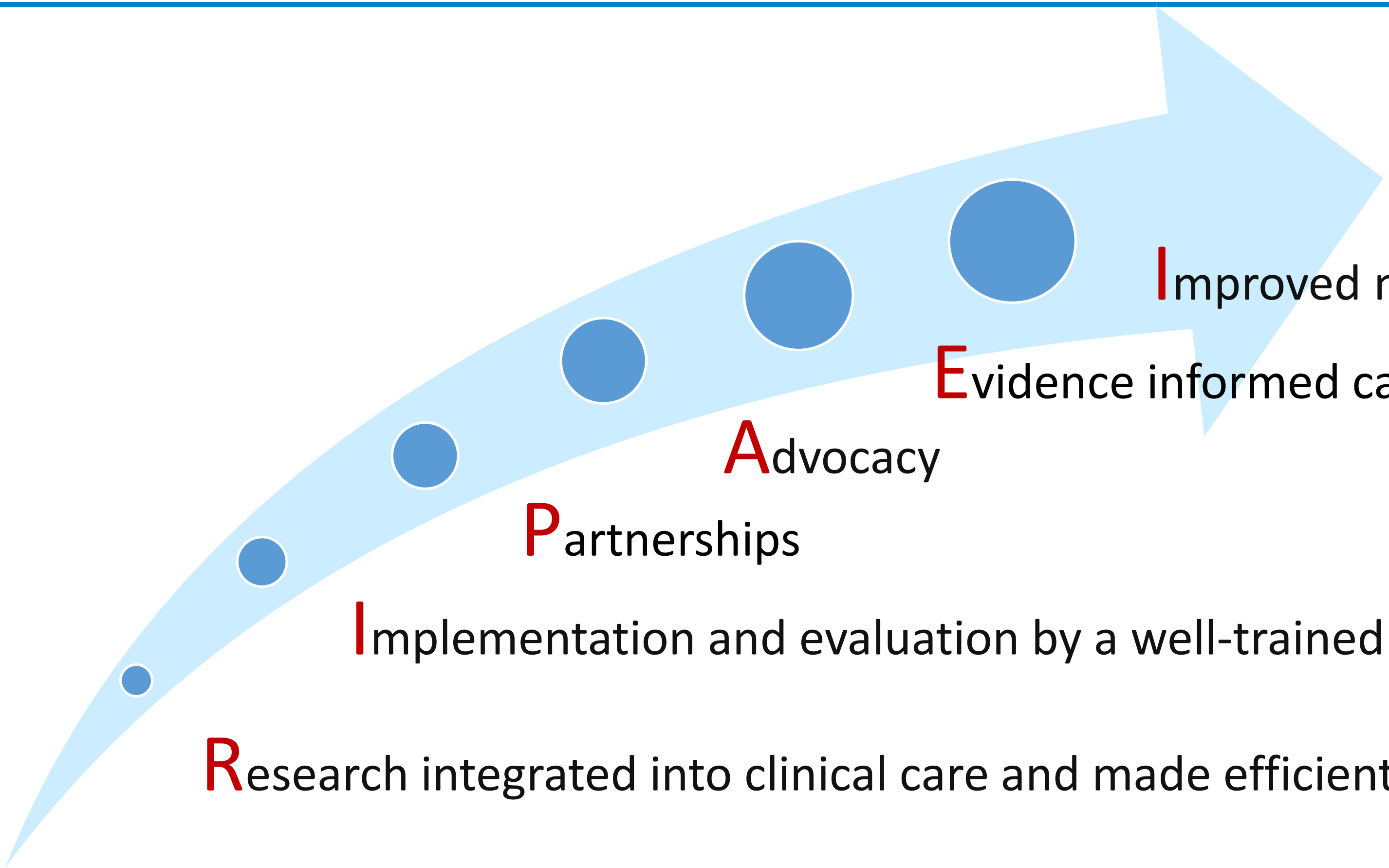
Data cleaned

Deposited in National Neonatal Research Database

Non-trial patient data extracted for generalisabilty evaluation

Participant data extracted into trial specific data file

An improvement pipeline for newborn and population health



Improved newborn health

Evidence informed care and policy

Advocacy

Partnerships

Implementation and evaluation by a well-trained workforce

Research integrated into clinical care and made efficient

Acknowledgements and thanks

With thanks to the babies, their parents, our collaborators and research funders

Agenda – Embracing Research Culture

9:45 a.m.	**Session I:** *Embracing a Research Culture* GERRI BAER (FDA) & MARY SHORT (LILLY), CO-CHAIRS *Fostering a Culture of Research to Improve Outcomes* KELLY WADE (CHILDREN'S HOSPITAL OF PHILADELPHIA)
10:15 – 10:45 a.m.	COFFEE BREAK
10:45 a.m.	**Session I Panel:** *Fostering a Culture of Research: Beliefs, Strengths/Barriers, Needs, INC role/opportunity for Establishing a Research Culture* MEHALI PATEL (BLISS) JENNIFER CANVASSER (PREEMIE PARENT ALLIANCE) YANNIC VERHAEST(EFCNI) CATHERINE SHERWIN (UNIVERSITY OF UTAH) CAROLE KENNER (COINN) WAKAKO EKLUND (NANN) JORDI LLINARES-GARCIA (EMA) NATHALIE SEIGNEURET (IMI) CHRISTINA BUCCI-RECHTWEG (NOVARTIS)

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CRITICAL PATH INSTITUTE
International Neonatal Consortium

Session I: Embracing a Research Culture

Co-Chairs:
Gerri Baer, MD
Mary A. Short RN,MSN

CRITICAL PATH INSTITUTE

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

We have a dream

“Every newborn admitted to the NICU will enroll in a study protocol to optimize outcomes;

The definition for our most important outcomes will be the same worldwide;

We will collect standardized data on all infants, and the databases will be shared, harmonized and readily searchable;

We will be able to easily examine survival and outcome based on region of the world and adopt best practices;

We will have established normal lab values based on birthweight, gestational age and postnatal age.”

-Jon Davis

Our dream requires a culture that embraces research.

What is a “culture?”

a) the integrated pattern of human knowledge, belief, and behavior that depends upon the capacity for learning and transmitting knowledge to succeeding generations

b) the customary beliefs, social forms, and material traits of a racial, religious, or social group; also : the characteristic features of everyday existence (as diversions or a way of life) shared by people in a place or time <popular culture> <southern culture>

c) the set of shared attitudes, values, goals, and practices that characterizes an institution or organization <a corporate culture focused on the bottom line>

d) the set of values, conventions, or social practices associated with a particular field, activity, or societal characteristic

From Merriam-Webster.com (accessed 8/29/2016)

Examples of Culture Change in Neonatology

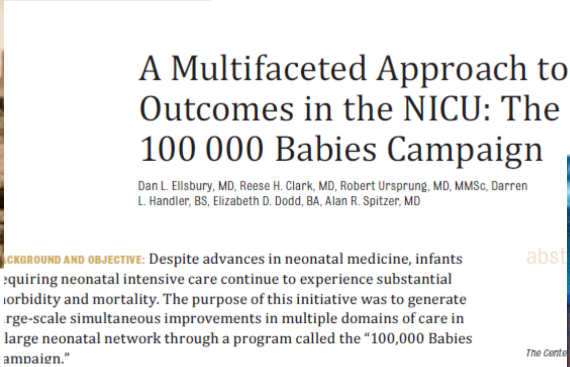
- From parents peering in the windows to Family-Centered Care and parent participation in NICU caregiving

Akron Children's 1970

[redacted]

Examples of Culture Change in Neonatology

- From the “Wild West” to thousands of NICUs doing quality improvement



A Multifaceted Approach to Improving Outcomes in the NICU: The Pediatrix 100 000 Babies Campaign

Dan L. Ellsbury, MD, Reese H. Clark, MD, Robert Ursprung, MD, MMSc, Darren L. Handler, BS, Elizabeth D. Dodd, BA, Alan R. Spitzer, MD

CKGROUND AND OBJECTIVE: Despite advances in neonatal medicine, infants equiring neonatal intensive care continue to experience substantial orbidity and mortality. The purpose of this initiative was to generate rge-scale simultaneous improvements in multiple domains of care in large neonatal network through a program called the “100,000 Babies ampaign.”

abst

The Cente

2016 Annual Quality Congress and NICQ Symposium
September 9-12, 2016
Sheraton Grand Chicago

Registration is still open!

Register now for the 2016 VON Annual Quality Congress and NICQ Symposium in Chicago

ongress sium

A Community of Practice

Cochrane Neonatal Review Group

VON News

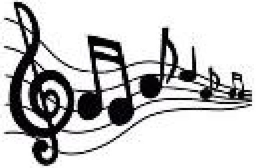
2016 AQC Dedication and Fundraising

Triple Continuing Education Accreditation

2015 Birth Year Reports are Available on Nightingale

The refrain: Research is needed!

- More units, babies, families, nurses, physicians engaged in research – are we “preaching to the choir?”



- Goals of this session:
  1. Learn about the successes, surprises, and limitations in one research-active NICU
  2. Listen to parents, nurses, regulators, industry and academic partners discussing perspectives on how to build research culture
  3. Panel and audience interaction to develop ideas for action
  4. Voting on potential workstreams to help bring research culture to the forefront in neonatology

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Embracing a positive research culture in the NICU

sharing stories from the trenches

Kelly C Wade, MD PhD MSCE

Children’s Hospital of Philadelphia, Newborn Care Network

Pennsylvania Hospital, University of Pennsylvania Health System

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SCIENCE MEDICINES HEALTH

CHOP Newborn Care at Pennsylvania Hospital

Pennsylvania Hospital

1751

Penn Medicine

- 1st Hospital in US 1751, Ob Care since 1929
- 5200 births with 925 NICU admissions
- 120 babies <30wk GA (40 babies <1 kg)
- >40 years of clinical research in neonatology
- 1st NICUs to join Vermont Oxford Network 1988
- Research site for surfactant and iNO studies
- Home of the bilirubin nomograms for ≥35wk GA
- NICHD Neonatal Research Network since 2011

The secret of success is constancy of purpose

When you are finished changing, you're finished

Honesty is the best policy

https://c1.staticflickr.com/5/4083/5180644307_78e2854212.jpg

Key Drivers promoting a positive research culture

1. **High level awareness that we still have work to do to improve outcomes**
   - Awareness of variations in practice / outcomes & importance of novel therapies that improved outcomes
   - Commitment to using research to improve outcomes with sufficient knowledge of clinical research
2. **Constant presence of research**
   - High visibility, broad range of eligibility, varied age at enrollment
   - WIDE range of perceived risk
3. **Research addresses challenges faced by all**
4. **Constancy of purpose – best outcomes, consistency in care**
   - ALL staff know this is a research active unit and must support the overall research effort
   - Agreement -best practices can be upheld regardless of research participation /randomization grp
   - Research stories provide continued dialogue about care of neonates and efforts to improve outcomes
5. **Family centered care – family engagement, and commitment to follow up**
   - Focus on building enduring relationships & trust while fostering open communication and dialogue
   - *Using stories to promote discussion & shared knowledge about advancing care & promoting safety*

Research Studies in the NICU –past and present

randomized, non-randomized, multicenter, single center, industry, NIH

- Surfactant
- Inhaled NO
- Palivizumab
- PROFIT prophylactic hydrocortisone
- NeoPAIN (morphine)
- Chlorthiazide ± spironalactone CLD
- Dopamine on organ perfusion
- Vitamin E for prevention of ROP
- Pulmonary function testing
- Serum bilirubin nomogram
- Gastric motility with suckling
- Early accelerated DC LBW infants

- Fluconazole prophylaxis
- Proparacaine for pain ROP exams
- Spontaneous Breathing Trial
- Canadian Oxygen Trial (COT)
- HFNC vs CPAP
- NIPPV vs CPAP
- Therapeutic hypothermia (longer, colder)
- Progression of sucking behavior
- Gene Targets for IVH
- ECHO vs clinical exam for PDA
- Biomarkers of meningitis - CSF
- Language check list

- Inositol for prevention of ROP
- **Donor milk vs formula**
- **Hydrocortisone for extubation**
- **TOP –transfusion threshold**
- **Incubator weaning trial**
- **SAIL sustained inflation vs PPV**
- **Therapeutic hypothermia -** premies
- **Pulse-ox & NIRS during RBC**
- **Effect of age at feeding initiation**
- **Health literacy in the NICU**
- **Early intervention referral**
- **Bundling and boundaries (PT)**
- **DC preparedness**

Research Studies in the NICU–Drug, device, other

- Surfactant
- Inhaled NO
- Palivizumab
- PROFIT prophylactic hydrocortisone
- NeoPAIN (morphine)
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- DC preparedness

Research addresses challenges faced by ALL

Physician/Infant Centered

- Donor milk study
- Hydrocortisone for extubation
- Inositol for prevention of ROP
- TOP –transfusion threshold
- SAIL –sustained inflation

NURSE Centered

- Effect of age at feeding initiation
- Incubator weaning trial
- Donor milk study
- Accelerated DC study

Device Centered

Respiratory / physical therapy

- COT –oxygen trial
- SAIL –sustained inflation vs PPV
- Therapeutic hypothermia-preemies
- Pulse-ox & NIRS during RBC

FAMILY Centered

- Health literacy in the NICU
- Early intervention referral at DC
- Bundling and boundaries
- Family readiness at DC
- Language check list

Everyone contributes to the positive research culture

RESEARCH STAFF

Physician, RN, nonRN coordinator

- Build & maintain relationships
- Build trust & foster communication
- Consent
- Perform research functions

NURSES

- Build & maintain relationships
- Build trust & foster communication
- Discuss families questions, concerns
- Describe their perspective of how research does (not) affects care

PHYSICIANS

- Build & maintain relationships
- Build trust & foster communication
- Stories of research and outcomes
- Variation vs evidence based care
- Describe/discuss research protocols
- Consent when time sensitive

FAMILY

- Build & maintain relationships
- Build trust & foster communication
- Willingness to discuss research
- Ask questions / Share thoughts
- Commit to follow up
- Family advisory council

Constancy of purpose - research active units

seek the best outcomes & consistency in care for ALL

- COT trial - Standardized oxygen targets throughout unit, better adherence to targets, enhanced information output from pulse oximeters
- Hypothermia trials–standardized availability, updated EEG/MRI, certified neuro-examiner
- Milk Trial – Improved availability of donor milk, enhanced commitment maternal milk, standardized milk fortification and preparation
- NIPPV & High Flow Trials – optimized use of non-invasive respiratory support
- Feeding Studies – closer look at feeding practices in unit
- TOP trial – better adherence to transfusion protocol and less frequent lab tests
- Heath Literacy Study– highlight importance of health literacy

Family centered care – family engagement

using stories to promote discussion & share knowledge about advancing care, promoting best outcomes, and safety

- **Stories can celebrate past research that advanced care**
  - Treatment RDS before surfactant, PPHN before inhaled nitric oxide
  - Delivery room care and optimization of non-invasive ventilation
- **Stories can share understanding of safety monitoring in research**
  - Awareness that sometimes our routine practice is not as good as we thought
  - DSMB, willingness to close trials, maintaining physician autonomy in decisions
- **Stories of research can answer families questions & promote discussion**
  - Why not just give surfactant to all extremely low birth weight infants?
  - Why would you wrap my baby in plastic?   Isn't caffeine dangerous?
- **Stories about how research oriented culture can improved care for all**
  - More unit based protocols for providing a consistent standard of care
  - Improved access to technology and its use from protocol guidance
  - More conversations with families about care decisions and problems of prematurity

Why should I put my baby at risk?

- Babies are already at risk due to prematurity or illness
- Improvements in survival & outcomes come through rigorous research
- Discuss what is NOT known and variation in practice
- Teach families about origins of research studies chosen for the NICU
- Research standardizes care practice, beyond the intervention
- Research offers staff advanced training and access to experts
- Teach families about safety monitoring in clinical trials
  - More oversight of care
  - Maintain the authority to do the right thing, even in research
  - All on the same team striving for best outcomes, whether or not you participate
  - Share stories of surprises revealed through research

Lessons learned along the way

- Importance of safety in research
  - Experience of a closed trial gives stories of the DSMB function & safety monitoring
  - When studies close early, the initial question remains unanswered
  - Important that physician maintains autonomy for treatment decisions
- Sometimes our standard of care was inferior when studied in a trial
- Unique challenges in studies that run for long period of time
  - Families can forget their infant is participating in research
  - Can receive mixed messages about whether care is for ongoing disease or research
  - Need continued engagement with families, families want updates
  - Avoid study fatigue & change in equipoise over time among care providers
- Families are interested in strategies to make research infant friendly
- Families are interested in origins of research protocol and outcomes

Limitations in a research active NICU

- Research saturation
  - commitment to a network research, decline in industry sponsored trials
  - difficult to bring in new studies
  - difficult if studies will not allow co-enrollment
  - Infants are eligible for many studies (VLBW)
- Difficulty obtaining consent in studies requiring time sensitive enrollment
- Study fatigue and maintaining equipoise among physicians and nurses
- Ensuring support for infants/families not participating in research
- Difficulty maintaining consistent funding for research support staff
- Academic promotion can be difficult, most faculty are clinical
- Retirement of long standing faculty and coordinators

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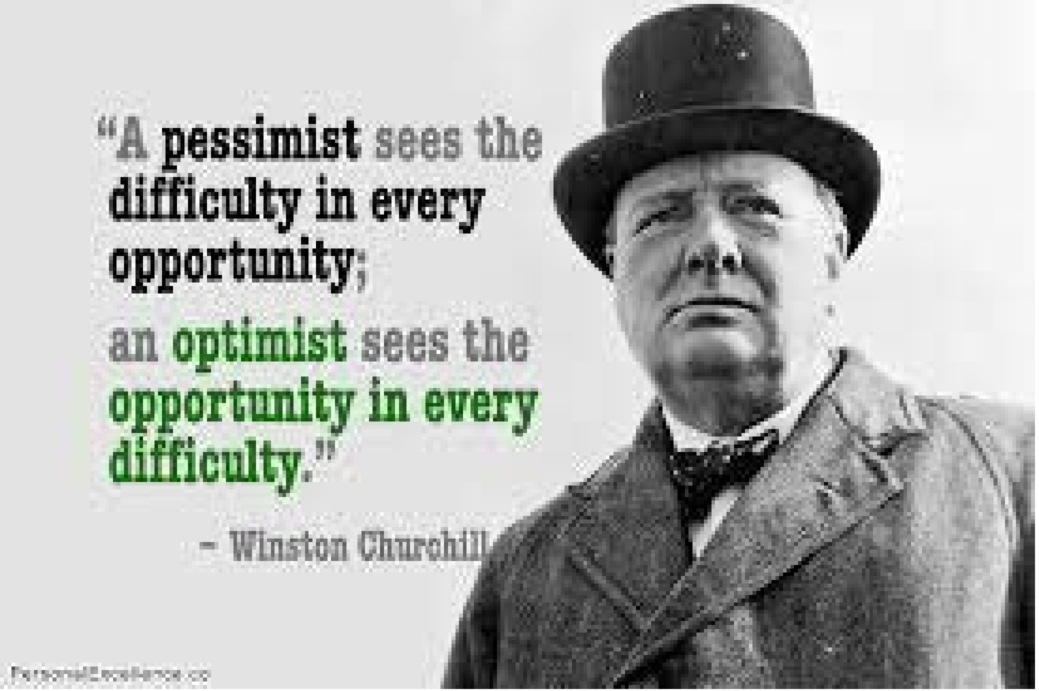
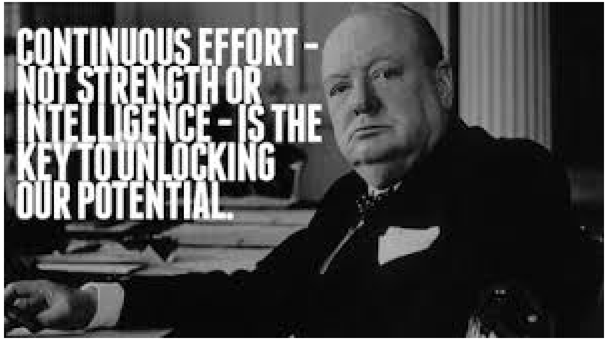
For myself I am an optimist



"A pessimist sees the difficulty in every opportunity; an optimist sees the opportunity in every difficulty."

- Winston Churchill



CONTINUOUS EFFORT - NOT STRENGTH OR INTELLIGENCE - IS THE KEY TO UNLOCKING OUR POTENTIAL.

Thank you

http://personalexcellence.co/quotes/4128, http://likesuccess.com/959344

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Yannic Verhaest (EFCNI)

- Representing EFCNI (European Foundation for the Care of Newborn Infants) or the European voice of parents
- Chairwoman of the Dutch parents association VVOC (Belgium)
- Mother of two girls, one born at 30 weeks
- Psychotherapist
- Infant Mental Health specialist
- Researcher for 15 years at the University of Leuven: Process-outcome and Follow-up studies on the psychotherapy unit for personality disorders KLIPP, UPC KU Leuven

Challenges for Parents

- How can parents process information when they are in a state of trauma and their capacity to mentalise is down?
- Not only the health but the survival of their baby fills their minds.
- Can parents freely say yes or no to research when the researcher is part of the team that saves their child?
- Once home, parents try to let go of the medical world. Follow-up research reactivates the NICU period
- …

Research as a way of taking care of the parents?

Parents give us information, data, their baby! for our research.

What do we give them? What can help them?

How can we find ways that research is nourishing both researcher and parents, so that parents and their baby feel cared for and feel engaged in the research?

Possible Solutions?

An environment of trust

Feeling supported by the team

Feeling they are not a number, but a family with it’s own story, background, culture,…

Feeling someone cares about them and is willing to listen

Giving honest and complete information in a way that can be processed

Feeling gratitude from the team for their participation

Be kept informed about further developments and the results of research

…

Voting Slide – Research Culture

Considering both impact and feasibility, which of the following projects is your **first** choice?

1. Enhancing **general communication and public relations** around neonatal research
   - Publicize the reasons neonatal research is important, research success stories, patient/parent rights, patient protections, provide resources for families
   - Tools for education, communication, and consent
   - Create document or handbook for NICUs that would like to develop research programs; includes key elements of successful units/groups
2. **Family-centered research** embedded in family-centered NICU culture – facilitating parents' ability to choose research that is best for their child/family
   - Process for keeping families updated on the results of research
3. Increased involvement of former NICU parents and graduates, nurses, other **multidisciplinary team members in the design and planning of research**
4. Enhance nursing education in regards to research
5. "Walk-in Option A" (offered up by audience)
6. None of the above

Voting Slide – Research Culture

Considering both impact and feasibility, which of the following projects is your **second** choice?

1. Enhancing **general communication and public relations** around neonatal research
    - Publicize the reasons neonatal research is important, research success stories, patient/parent rights, patient protections, provide resources for families
    - Tools for education, communication, and consent
    - Create document or handbook for NICUs that would like to develop research programs; includes key elements of successful units/groups
2. **Family-centered research** embedded in family-centered NICU culture – facilitating parents' ability to choose research that is best for their child/family
    - Process for keeping families updated on the results of research
3. Increased involvement of former NICU parents and graduates, nurses, other **multidisciplinary team members in the design and planning of research**
4. Enhance nursing education in regards to research
5. "Walk-in Option A" (offered up by audience)
6. None of the above