# PEDDCReN

## Paediatric European Digestive Diseases Clinical Research Network Project













## Aims

 To support the development, funding and planning of high quality, trials in children with GHN disorders.

- Early engagement with industry in study plans
- Support investigator led studies, (eg sign posting to funds, feasibility identification of collaborators)
- Model PIPS, agreed outcome measures
- Feasibility data etc

**PEDDCReN - Paediatric European Digestive Diseases Clinical Research Network** 













2

## Key partnerships

Led by British, Irish and Dutch Societies of Gastroenterology

**ESPGHAN** - European Society of Paediatric Gastroenterology, Hepatology and Nutrition

**ENPR-EMA** - European Network of Paediatric Research – European Medicines Agency















- Web based secure software
- Electronic surveys and databases
- Widely used



**PEDDCReN - Paediatric European Digestive Diseases Clinical Research Network** 













4

## PEDDCReN

## Expertise, resources and interest

- 71
   investigators/
   centres
- 61 Cities
- 24 Countries















## Register – Key points

### Interest and Facilities

- All willing to participate in PEDDCReN
- 56% willing/ able to take on phase I or II studies
- 37% Dedicated paediatric clinical research facilities available on site
- 34% Access to a research nurse

### Investigator experience

- 33% had been Chief Investigators for their country
- 58% had been Principle Investigators in their hospital
- 27% had experience of phase I or II studies













## Register – Key points

- Approx 1000 NEW IBD patients per year
- Very Rare Diseases (out of 71 centres)
  - 17 units had patients with congenital enteropathy
  - 11 Congenital transport defect
  - 41 Polyposis syndromes
  - 28 Chronic intestinal pseudo-obstruction













## PEDDCReN Outcome PIBD Horizon 2020 Bid

- 6,000,000 euros
- PEDDCReN WP 6
  - Inception cohort of 300 IBD patients per year
  - Pan European safety monitoring of rare complication of IBD













## PEDDCReN and industry/CROs

- Access to experienced and motivated investigators
- Key stakeholders academics, clinicians, professional societies (ESPGHAN), EMA
- Pan European feasibility
- Information on patient populations
- Ability to perform feasibility and prospective identification of patients













## GI Liver Rare diseases proposal

- Prospective study of incidence and prevalence and incidence of rare and very diseases (Redcap)
  - ? Liver
  - ? Intractable diarrhoea
  - ? Pseudo-obstruction
- Develop national guidelines for investigations eg infant diarrhoea
- Deep phenotype and genotype information













## Rare diseases proposal

- Date supports feasibility of trials (Redcap)
- Plan intervention studies
- Improve care (early identification, better quicker diagnosis, new treatments)













## How: Monthly Ecard



### **E-reporting card**

Please complete the E-card below to indicate whether or not you have seen cases for APRIL 2015. The reporting instructions for APRIL 2015 are available to download by clicking here(PDF, 150KB)

The BPSU Spring bulletin is now available to download here @(PDF, 982KB)

The BPSU Annual Report 2013-14 can be viewed here

#### Surveillance of Acute Rheumatic Fever and Behcet's syndrome to commence next month

Surveillance on acute rheumatic fever (ARF) is due to start next month. Dr Mary Salama, Birmingham Children's Hospital, was awarded the BPSU Tizard Bursary to analyse ARF and how it affects 0-16 year olds. Download the flyer here (PDF, 299KB)

Surveillance of Behcet's syndrome is also due to commence and will run for 13 months with a one year follow-up. The study is led by Dr Clare Pain of Alder Hey Hospital and the study team hope to ascertain the incidence and prevalence of Behoet's syndrome. Download the flyer here (PDF 284KB)

Have you seen any cases in the month APRIL 2015?

\* must provide value

🔾 Yes	
$\bigcirc$ No	

~

V

~

~

~

#### Cases to report

Please specify in the appropriate box the number of cases seen:

To access the study information page on our Web site, click on the condition name.

1. HIV Infection & Perinatal HIV Exposure

2. Progressive Intellectual & Neurological Deterioration
3. Congenital Rubella
4. Group B Streptococcal Disease < 90 days

5. Enterovirus & Parechovirus Meningitis < 90 days

### **PEDDCReN - Paediatric European Digestive Diseases Clinical Research Network**







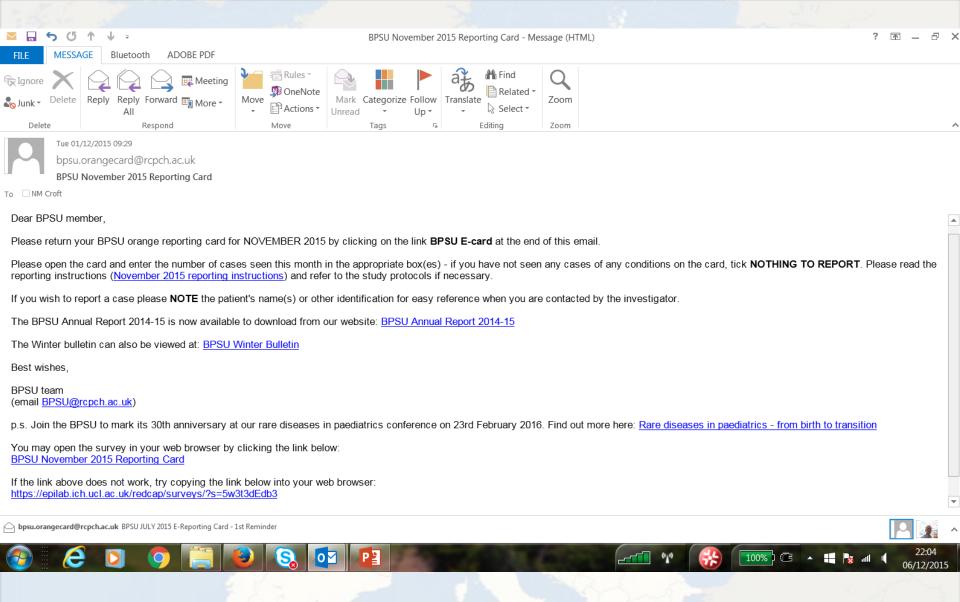




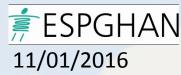
Resize font

A A





### **PEDDCReN - Paediatric European Digestive Diseases Clinical Research Network**











University of London

## Find patients

## BPSU E-reporting card

Please complete the E-card below to indicate whether or notypou have seen cases for APRIL 2015.

The reporting instructions for APRIL 2015 are available to download by <u>clicking here</u>(PDF,150KB) The SPSU Spring bulletin is <u>now available to download here</u>(PDF,962KB)

The SPSU donual Report 2019-14 can be viewed here

Surveillance of Acute Rheumatic Fever and Behoet's syndrome to commence next month

Surveilance on acuse rheumatic fever (ARP) is due to contractment. Dr Mary Salama, Briningham Children's Hoopial, was awarded the SPSU Titand Bursary to analyze ARP and how haffects 0-16 year olds. <u>Download the fyer here</u> (PDR/20045)

Surveilance of Sehcats syndrome is also due to commence and  $\phi$  will run for 15 monte with a one year bilow-up. The study is led by Dr Clare Pain of Alder Hey Hosgisl and the study sam hope to accertain the incidence and prevalence of Sehcats syndrome. <u>Download the fyer here</u> (PDF, 264/6)

0

0

#### E-oard

Have you seen any cases in the month APIGL 20157 Interpretide value

Resta font

616

#### Cases to report

Please specify in the appropriate box the number of cases seen

To access the study information page on our Web site, click on the condition name.

1.11Winfection & Perivatal 11WExposure	
2. Progressive intellectual & Neurological Deterioration	
3. Congenital Rubella	
4. Broug R. Rhophanami Chanan - Miring.	
A.Polecultus, S. Parashadras, Modigli, - Midge,	
A Pering Real Technic Clicky	
7. Mak Band Richals	
1. Type 2 Distantes	

#### Thank you for reporting a case

Please make a note of the case details for future reference.

Please keep a record of the report as an aide memoir. The Study Investigator will contact you shortly with a questionnaire.

#### Change of details

Please inform us if you have rebred or no longer have dimical work.

It you have moved, or would prefer emails to be sent to an alternative address, please include your new contact details :-

Data protection: The DPUJ maintains a fact of contact details for clinicians meaking the Grange Cand and we may contact you periodically to confirm these. You may also contact the DPUJ to ask to change cand format onto be removed from our fac.

Save & Return Later

### **PEDDCReN - Paediatric European Digestive I**





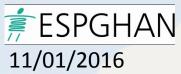


			LUI	

Strueillance or	Sadas York: A   A		
BPSU E-reportin	g card		
Plasse complex the G-card below to holicate whether or nonycorhave seen cases for APP The reporting instructions for APPL 2015 are available to download by <u>clothing here</u> (PDP, The PDPL Spring below is in <u>non-available to download here</u> (PDP, Stolica) The EPPL Jonnal Report2015-14 can be <u>viewed here</u>			
The weak of house Report of the Cambo <u>environment</u> Surveillance of Access Rheumatic Fewer and Dehcarks synchrone to commence next more Surveillance and access rheumatic two (CRP) is due to commence and the Surveillance Borcary to margies ARF and how tarbace - 64 your olds. <u>Convicted the synchron</u> (RSS) Surveillance of Schoeft synchrone is also due to commence and (welling for 15 monthe to deter Hey Haceford and the study sum hoge to accessible the Accelerate and (we silvers of			L
E-oard			
Here you seen any cares in the month APAE, 2015? "nutryouble value	1.11Winfection & Perinacal IIIV Exposure		
Cases to report	2. Programive intellectual & Neurological Deterioration		
Please specify in the appropriate box the number of cases are To access the aludy information page on our Web alls, dick	2. Congenital Rubela		
1.11MInfection & Performant II/VExposure			
2. Prograssive Institutual & Neurological Desertoration	4. On say IC No spinorend Channe - 10 step.		
3. Congenital Rubella			
5.00 sap 8.80 spherosed Theorem 20 sign	A Pole sales. A Por salasies. Meinglis - Meing.		
<u>k Pairada a K Pairada a Balagia - Bilaga</u> <u>K Pairaga Kuni Tanaksin dil Aga</u>	A Participa Charl Stree Julies 20 styr.		
1. Web Theord Window			
<u>1. Type 2 Division</u>	7. Note Band Witten.		
I hank you for reporting a case Please make a note of the case details for future refe Please have a record of the record as an ada memori. The Stu	8. Type 2 Distante.		
questionners. Change of details	Thank you for reporting a case		
Fleese inform us if you have retired or no longer have clinical work. If you have moved, or would prefer emails to be sent to an attemative address, please include your new contact details here.	Flease make a note of the case details to Please keep a record of the report as an aide me		ited you shortly with a
Data presection: The DP3U maintains a list of contact details for clinicities confirmments. You may also contact the DP2U to address change and form			
Submit Sana & Ratum La			Barts and The London School of Medicine and Dentistry
	BRITISH SOCIETY OF GASTROENTEROLOGY European Network of Paediatric Research at the European Me	rdicines Agency	

Phenotype information collect and store biobank specimens

autorating so Jan	A.
• BPSU • Orange Electronic	Reporting Card
1986-2016 Jorange Liectionic	Reporting Card
The service of the se	
thans of	
Please complete the E-card below to indicate whether or not you	have seen cases for JULY 2015.
The reporting instructions for JULY 2015 are available to downlo	ad by clicking here (PDF, 150KB)
The BPSU Annual Report 2014-15 can be viewed here.	
The Spring edition of the BPSU Bulletin can be viewed online.	(PDE 982KB)
nie opinių canon or ne broo bancan dan be <u>neneo oninie</u> . (	( 51, 00210)
Name of responsible consultant	
* must provide value	
Hospital name	
Consultant telephone number	
Consultant email address	
	Work email preferred
Which laboratory notified the screening result?	~
	Please choose one answer
Details of child	
Date of birth * must provide value	Today D-M-Y
must provide value	
NHS number / CHI number / Health & Social Care	
Number	
* must provide value	
Hospital Number	
Postcode of residence (first half only)	
i ostoble of residence (in st han only)	
Sex	
* must provide value	<b>v</b>
Ethnic group	~
	Codes in brackets are those used on the newborn bloodspot card.
Country of birth (if known)	
County of birdi (ii known)	
First presentation and clinical features	
How was the child first detected?	O Routine newborn screening
	Routine newborn screening     Tested because of family history
	Clinical signs/symptoms
	O Other
	O Other



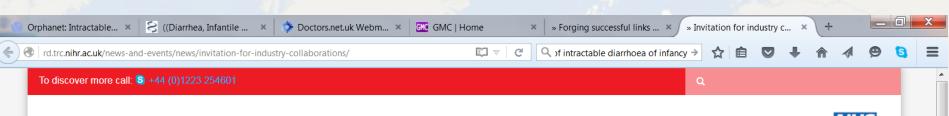












#### Rare Diseases Translational Research Collaboration

### National Institute for Health Research

#### About us

RD-TRC Meetings

Training and capacity building

Vacancies

Funding call

#### Themes

Informatics

Patient, carer and public involvement

Collaborate with RD-TRC

Funding call

Training and development

News and events

Contact us

### Invitation for industry collaborations

Published in News on 17 December 2014

#### **CLOSING DATE FOR APPLICATIONS 11 DECEMBER 2015**

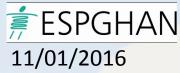
The NIHR Rare Diseases Translational Research Collaboration is inviting applications to establish industry–collaborative early translational (experimental medicine) research projects in rare diseases. These projects will create and support collaborative research between investigators working within NIHR-funded research infrastructure and industry partners. Specifically, the invitation aims to fund projects that generate in-depth phenotypic information to develop new treatments and speed up diagnosis.

NIHR RD-TRC funding is available to academic investigators specifically to support in-depth phenotyping. Where a proposal involves other research methodology (for example, a clinical trial or acquisition of genomic data) only the phenotyping aspects of a study are eligible for funding by the NIHR RD-TRC. Other research costs must be met by the industry partner or another appropriate funding source. Lead applicants must be based within an NIHR Biomedical Research Centre, Biomedical Research Unit or Clinical Research Facility. Applications require a named industry collaborator and need to state the contribution of the industry partner to the project.

Maximum RD-TRC funding for in-depth phenotyping of £200,000 is available over two



### **PEDDCReN - Paediatric European Digestive Diseases Clinical Research Network**











100%) 🗁 🔺 💾 🍢 📶 🍕

08:08

07/12/2015

Summary and questionsEach disease is rare/very rare

 Is there a forum for pharma to collaborate funding across multiple diseases in this area ?

• Is there a way to apply for funds as a group rather than to each individually?

