European Reference Network
for rare or low prevalence complex diseases

Network Inherited and Congenital Anomalies (ERNICA)

PATIENT - CLINICIAN PARTNERSHIP

ERNICA

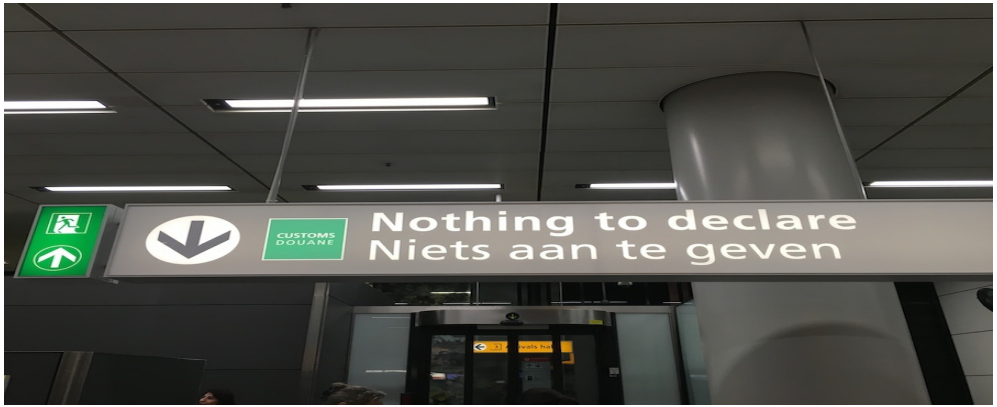
ERN Inherited and Congenital Anomalies (Intestinal tract)

Brussels,
22nd November 2018

Graham Slater
Prof. René Wijnen

We have no actual or potential conflict of interest

in relation to this programme/presentation



SUPERINTENDENT:
H. HEARDMAN

TEL. NOS.
SWINTON 2021-2-3

PENDLEBURY,

NR. MANCHESTER.

JMS/MS.
53/8275.

13th January, 1954.

Dr. Kelly,
Stonehouse,
Ribchester,
Nr. Preston.

Dear Dr. Kelly,

r[redacted]born 27.11.53.
2, Windsor Avenue, Longridge,
Preston.

[redacted] was admitted under the care of Mr. Jolleys on the 2nd December when he was four days old, with a history of vomiting and cyanosis when feeding was attempted the previous day and on each repetition of this. He had lost 1 lb. in weight and had been noticeably 'bubbly' in his throat since birth.

A catheter was passed down his oesophagus under screening control and a small quantity of opaque medium was introduced, showing an oesophageal atresia ending blindly, and gas present in the stomach indicated that there was a tracheo-oesophageal fistula. As his chest condition was very unsatisfactory on admission he was treated for 24 hours with streptomycin and penicillin, continuous suction from the mouth and pharynx and he was rehydrated with subcutaneous fluid. The following day Mr. Jolleys carried out a thoracotomy, the tracheo-oesophageal fistula was closed and the track excised, and the blind end of the upper oesophagus opened and anastomosed to the lower oesophagus. He was fed by gastrostomy tube for a fortnight after which he took feeds satisfactorily by mouth and made an uneventful recovery. He had a slight cough which is likely to persist for several months. At the time of his discharge on the 6th January an appointment was made for him to attend Dr. Hesling's clinic at Preston Infirmary.

Yours sincerely,

J. McSmellie

J.M. SMELLIE.
Senior House Surgeon.

‘FROM YESTERDAY TO TOMORROW’

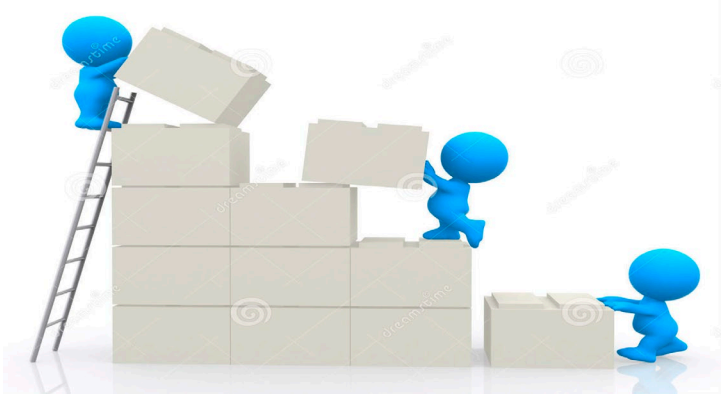
From

LIMITED COMMUNICATION

-> PATIENT ADVOCACY

-> COLLABORATION

-> ‘CO-BUILDING’



Patient-Clinician Partnership in ERNICA

Key factors contributing to success

- Coordinator's philosophy (regarding patient engagement)
- Patient representatives engage 'professionally'
- Prior experience of collaboration/engagement with support groups
- Mutual respect
- Trust
- Recognition that patients are a valuable resource in developing and - crucially - in implementing ERNICA's deliverables

Patient-Clinician Partnership in ERNICA

Key factors contributing to success

- **Coordinator's philosophy** (regarding patient engagement)
  - I am a father of 5 children, and 2 born with a RD
  - Patient and family centered care (PFCC)

    more efficient
  - Patients and parents will be recognised →

    higher perception, better experience ->> satisfaction
  - ERN: **Share**, Care and Cure

ERNICA
Work-packages involving patients

Standards of Care

- ✓ Guidelines: diagnostic, treatment and follow-up
- ✓ Quality care standards and ‘The Patient Journey’ (a team of 6, 50/50 patient reps/clinicians, led by the patients)
- ✓ Registries
- ✓ PROMs

J Pediatr Surg. 2018 Apr;53(4):610-615. doi: 10.1016/j.jpedsurg.2017.05.033. Epub 2017 Jun 9.

A patient led, international study of long term outcomes of esophageal atresia: EAT 1.

Svoboda E[1], Fruithof J[1], Widenmann-Grolig A[1], Slater G[1], Armand F[1], Warner B[1], Eaton S[2], De Coppi P[2], Hannon E[3].

Author information

Abstract

INTRODUCTION: Long term outcomes of esophageal atresia (OA) are poorly understood. The Federation of Esophageal Atresia and Tracheo-Esophageal Fistula support groups (EAT), a collaboration of patient support groups aimed to define patient reported long term outcomes and quality of life (QoL) in a large international cohort of OA patients.

METHODS: Questionnaires were designed focusing on patient/parent reported outcomes including surgical history, current symptomatology and quality of life. Members of support groups within EAT were invited to complete questionnaires electronically via SurveyMonkey®.

RESULTS: 1100 patients from 25 countries responded to the questionnaire and 928 were analyzed. 80% had type C anatomy, 19% type A and 1% type E. Patient ages were <5 years (42%), 5-10 years (26%), 11-17 years (16%) and 18 years and older (16%). 49% of all patients reported previous dilatations which was similar across age groups. Reflux symptoms affected 58% of patients and persisted into adulthood. Dysphagia also persisted in the adult population with 50% reporting sometimes or often getting food stuck. Reflux was significantly more frequent in 'long gap' versus 'standard gap' patients ($p<0.005$). Respiratory symptoms and chest infections decreased in frequency with age. In children median SDS for height was -0.41 (IQR -1.4 to 0.67) and that for weight was -0.63 (-1.6 to 0.67). BMI in adults was 21.5. Quality of life was described as significantly affected by OA in 18% of patients while 25% reported no effect on QoL.

CONCLUSIONS: These results highlight the significant long term morbidity suffered by OA patients as children and into adulthood and suggest the need for quality transitional care. The patient designed and reported nature of the study gives a unique perspective to the results and emphasizes the benefits of collaboration.

Challenges

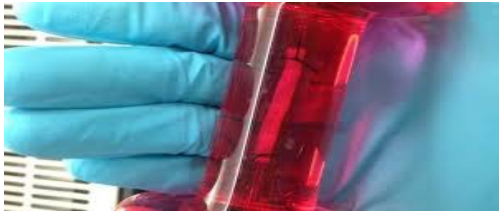
- Maintaining and strengthening the partnership
  - no complacency
  - embed patient involvement into the work of clinicans wherever possible and/or relevant (actually that's almost always!)
  - Different countries, different healthcare systems, different cultures -> also on PFCC / partnership

- Advocacy vs collaboration
  - the role of the patient representative - striking a balance
  - respecting the necessary professional (and ethical) processes and protocols associated with healthcare and related research

Working together for better outcomes

Developing (Co-building !) solutions

Supporting research to make
major breakthroughs in the treatment of congenital anomalies



Pediatr Res. 2018 Aug;84(2):181-189. doi: 10.1038/s41390-018-0063-3. Epub 2018 May 29.

Defining outcomes following congenital diaphragmatic hernia using standardised clinical assessment and management plan (SCAMP) methodology within the CDH EURO consortium.

IJsselstijn H[1], Breatnach C[2], Hoskote A[3], Greenough A[4], Patel N[5], Capolupo I[6], Morini F[6], Scharbatke H[7], Kipfmueller F[8], Ertresvag K[9], Kraemer U[10], Braguglia A[6], Wessel L[11], van Heijst AFJ[12], Moinichen I[9], Emblem R[9], Tibboel D[10]; CDH EURO Consortium Group.

⊕ **Collaborators (40)**

⊕ **Author information**

Abstract

Treatment modalities for neonates born with congenital diaphragmatic hernia (CDH) have greatly improved in recent times with a concomitant increase in survival. In 2008, CDH EURO consortium, a collaboration of a large volume of CDH centers in Western Europe, was established with a goal to standardize management and facilitate multicenter research. However, limited knowledge on long-term outcomes restricts the identification of optimal care pathways for CDH survivors in adolescence and adulthood. This review aimed to evaluate the current practice of long-term follow-up within the CDH EURO consortium centers, and to review the literature on long-term outcomes published from 2000 onward. Apart from having disease-specific morbidities, children with CDH are at risk for impaired neurodevelopmental problems and failure of educational attainments which may affect participation in society and the quality of life in later years. Thus, there is every reason to offer them long-term multidisciplinary follow-up programs. We discuss a proposed collaborative project using standardized clinical assessment and management plan (SCAMP) methodology to obtain uniform and standardized follow-up of CDH patients at an international level.

PMID: 29915407 DOI: 10.1038/s41390-018-0063-3

SOMA e.V.

EAT

cdh UK