

# How can we improve the evidence? How can ePAGs help?

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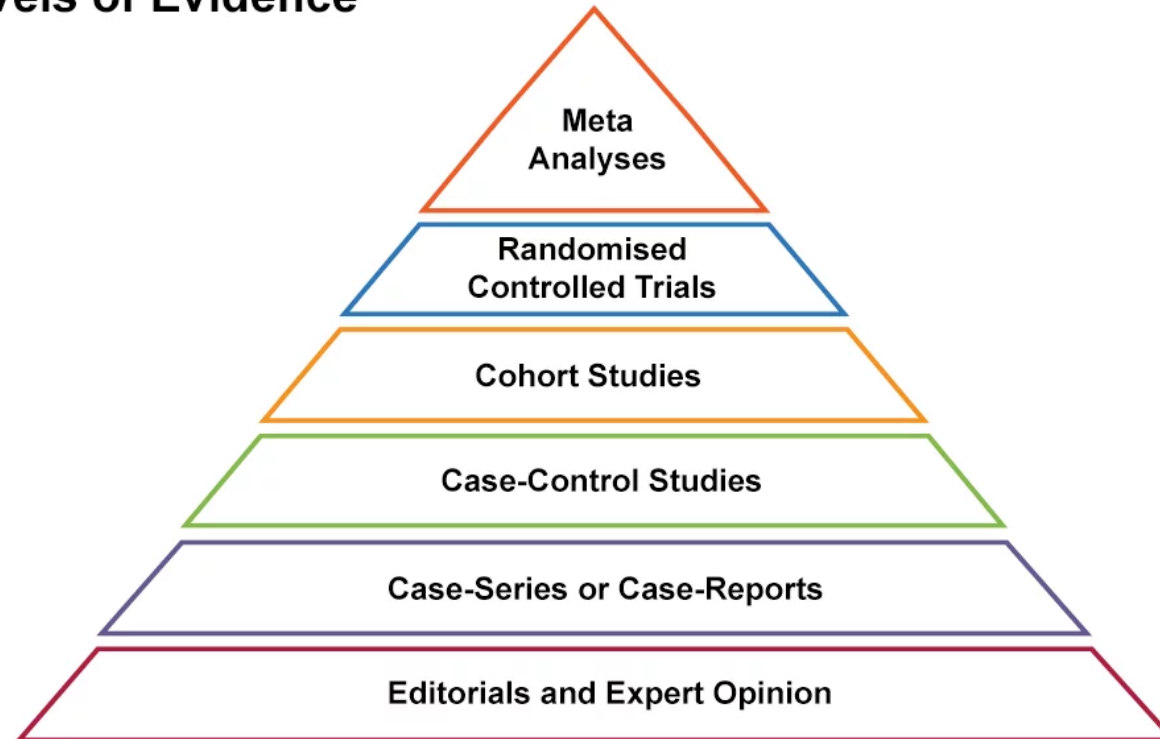
# Evidence-based medicine

## Levels of Evidence



# Evidence-based medicine

## Levels of Evidence



Common diseases  
(diabetes, stroke etc.)

Rare diseases



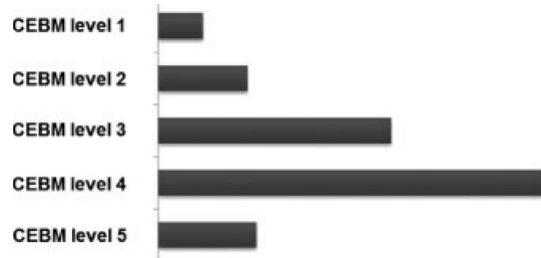
Whilst ensuring patients, parents  
and caregivers are at the centre



# ERNICA



Esophageal atresia  
2.4 / 10,000 live births



ePAG  
European Patient Advocacy Group



# ERNICA Consensus Statements

## ERNICA Consensus Conference on the Management of Patients with Esophageal Atresia and Tracheoesophageal Fistula: Diagnostics, Preoperative, Operative, and Postoperative Management

European Journal of  
Pediatric Surgery

Carmen Dingemann<sup>1</sup> Simon Eaton<sup>2</sup> Gunnar Aksnes<sup>3</sup> Pietro Bagolan<sup>4</sup> Kate M. Cross<sup>5</sup>  
Paolo Decoppi<sup>2,5</sup> JoAnne Fruithof<sup>6</sup> Piergiorgio Gamba<sup>7</sup> Steffen Husby<sup>8</sup> Antti Koivusalo<sup>9</sup>  
Lars Rasmussen<sup>10</sup> Rony Sfeir<sup>11</sup> Graham Slater<sup>12</sup> Jan F. Svensson<sup>13</sup> David C. Van der Zee<sup>14</sup>  
Lucas M. Wessel<sup>15</sup> Anke Widenmann-Grolig<sup>16</sup> Rene Wijnen<sup>17</sup> Benno M. Ure<sup>1</sup>

Consensus meeting over 2 days

- 82 items voted on
- controversy on 21 items

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## ERNICA Consensus Conference TEST

1 Should a nasogastric tube be routinely inserted as a diagnostic procedure in cases with suspected EA?

9 - Fully Agree

8

7

6

5

**Vote**

## ERNICA Consensus Conference TEST

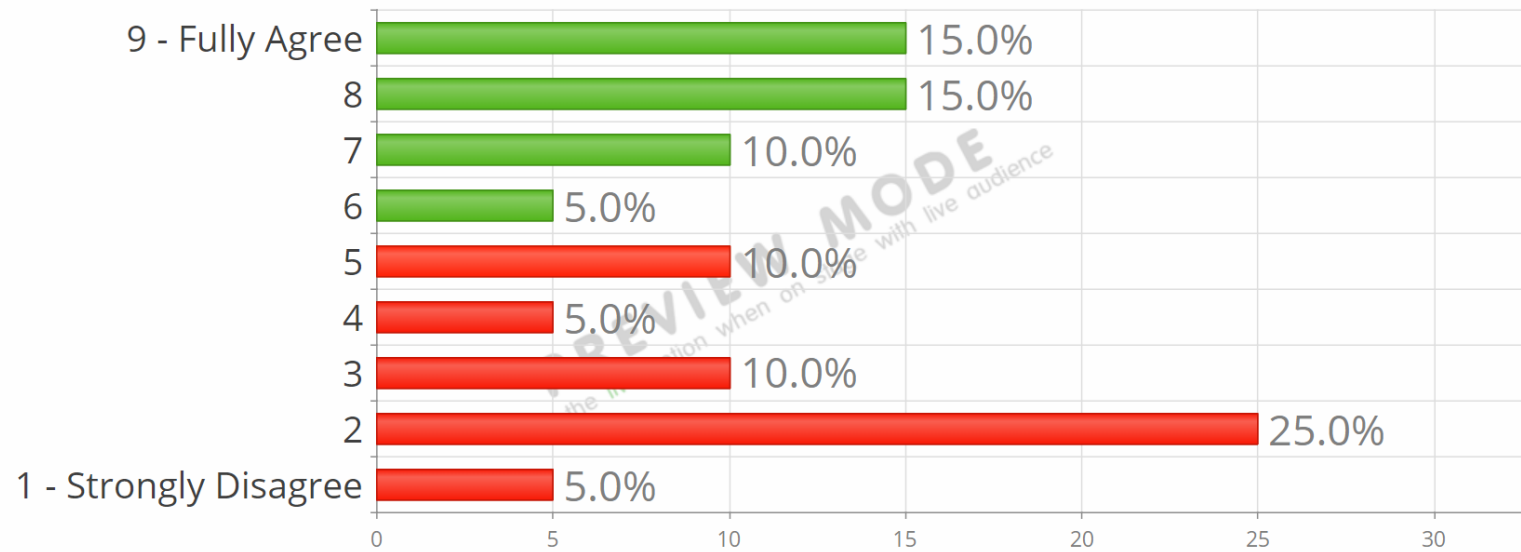
1 Should a nasogastric tube be routinely inserted as a diagnostic procedure in cases with suspected EA?

 3 2 1 - Strongly Disagree No expertise on this

**Vote**



# 1. Should a nasogastric tube be routinely inserted as a diagnostic procedure in cases with suspected EA?



20 users voted

ixvote  
own for free

Vote on [live.voxvote.com](https://live.voxvote.com) PIN: 85979

European Reference Network  
for rare or low prevalence complex diseases  
Network Inherited and Congenital Anomalies (INCA)



# ERNICA Consensus Statements

10	The maximum number of esophageal dilations for recurrent anastomotic strictures until a fundoplication should be considered is 5	+	83.3	15/18	8 [1-9]
11	Topical application of mitomycin C should be recommended as an option in patients with recurrent strictures	-	26.7	4/15	3 [1-9]
12	Intralesional/systemic steroids should be recommended as an option in patients with recurrent strictures	-	46.2	6/13	5 [3-9]
13	Customized stents /indwelling balloons should be recommended as an option in patients with recurrent strictures	+	100	14/14	8 [6-9]
14	24-hour-pH- or pH-impedance monitoring should be routinely used for monitoring children and adolescents with EA according a specific schedule	+	93.8	15/16	9 [5-9]
15	24-hour-pH- or pH-impedance monitoring should be routinely performed at time of discontinuation of antacid therapy	+	83.3	15/18	8.5 [2-9]
16	At least two additional pH studies should be routinely performed until transition	-	55.6	10/18	6 [1-9]
17	Endoscopies of the upper gastrointestinal tract should be routinely used for monitoring children and adolescents with EA according a specific schedule	+	94.4	17/18	9 [2-9]

Consensus yes/no

Agree/total votes

# ERNICA Consensus Statements

## ERNICA Consensus Conference on the Management of Patients with Long-Gap Esophageal Atresia: Perioperative, Surgical, and Long-Term Management

European Journal of  
Pediatric Surgery

Carmen Dingemann<sup>1</sup> Simon Eaton<sup>2</sup> Gunnar Aksnes<sup>3</sup> Pietro Bagolan<sup>4</sup> Kate M. Cross<sup>5</sup>  
 Paolo De Coppi<sup>2,5</sup> JoAnne Fruithof<sup>6</sup> Piergiorgio Gamba<sup>7</sup> Imeke Goldschmidt<sup>8</sup> Frederic Gottrand<sup>9</sup>  
 Sabine Pirr<sup>10</sup> Lars Rasmussen<sup>11</sup> Rony Sfeir<sup>12</sup> Graham Slater<sup>13</sup> Janne Suominen<sup>14</sup>  
 Jan F. Svensson<sup>15</sup> Joergen M. Thorup<sup>16</sup> Stefaan H. A. J. Tytgat<sup>17</sup> David C van der Zee<sup>17</sup>  
 Lucas Wessel<sup>18</sup> Anke Widenmann-Grolig<sup>19</sup> René Wijnen<sup>20</sup> Wilhelm Zetterquist<sup>21</sup> Benno M. Ure<sup>1</sup>

Consensus meeting over 2 days  
 - 97 items voted on  
 - controversy on 19 items



Table 9 Priorities for further research

	Domain	Topic
1	Diagnostics	Optimal approach for gap measurement
2	Definitions	Comprehensive definition of "long-gap esophageal atresia"
3	Esophageal reconstruction - Initial management before reconstruction	Counseling of parents (ideally including the involvement of patient support groups)
4	Esophageal reconstruction - Delayed primary anastomosis	Evidence for routine insertion of a transanastomotic tube
5	Esophageal reconstruction - Delayed primary anastomosis	Evidence for routine placement of a chest drain
6	Esophageal reconstruction - Lengthening techniques	Early and long-term outcome of different esophageal lengthening techniques
7	Esophageal replacement	Evidence for optimal surgical technique for esophageal replacement
8	Esophageal replacement - Gastric transposition	Early and long-term outcome after gastric tube formation as an option for esophageal replacement
9	Esophageal replacement - Gastric transposition	Evidence for insertion of a transanastomotic tube during gastric transposition
10	Esophageal replacement - Gastric transposition	Relevance of pyloroplasty (Mikulicz) during gastric transposition
11	Postoperative management	Evidence for routine postoperative contrast study of the esophagus before initiation of oral feeding
12	Postoperative management	Timing of the initiation of oral feeding
13	Follow-up	Duration of postoperative antacid therapy
14	Follow-up	Mode of tapering the postoperative antacid therapy
15	Follow-up	Evidence for peri-interventional antibiotic prophylaxis in balloon or semirigid dilatation for anastomotic stricture
16	Follow-up	Application of indwelling balloon dilatation, endoscopic knife, and surgical resection and reanastomosis in cases of recurrent anastomotic stricture

# How to take the evidence forward?

If all controversial items were converted to randomised controlled trials with 40 patients (very optimistic!), with an appropriate primary outcome, we would need to randomise:

- 840 EA-TOF patients  
(= almost every baby in EU for one year)
- 640 long-gap EA patients  
(= most long-gap in EU for ten years)

This is clearly completely impossible.

# Registries



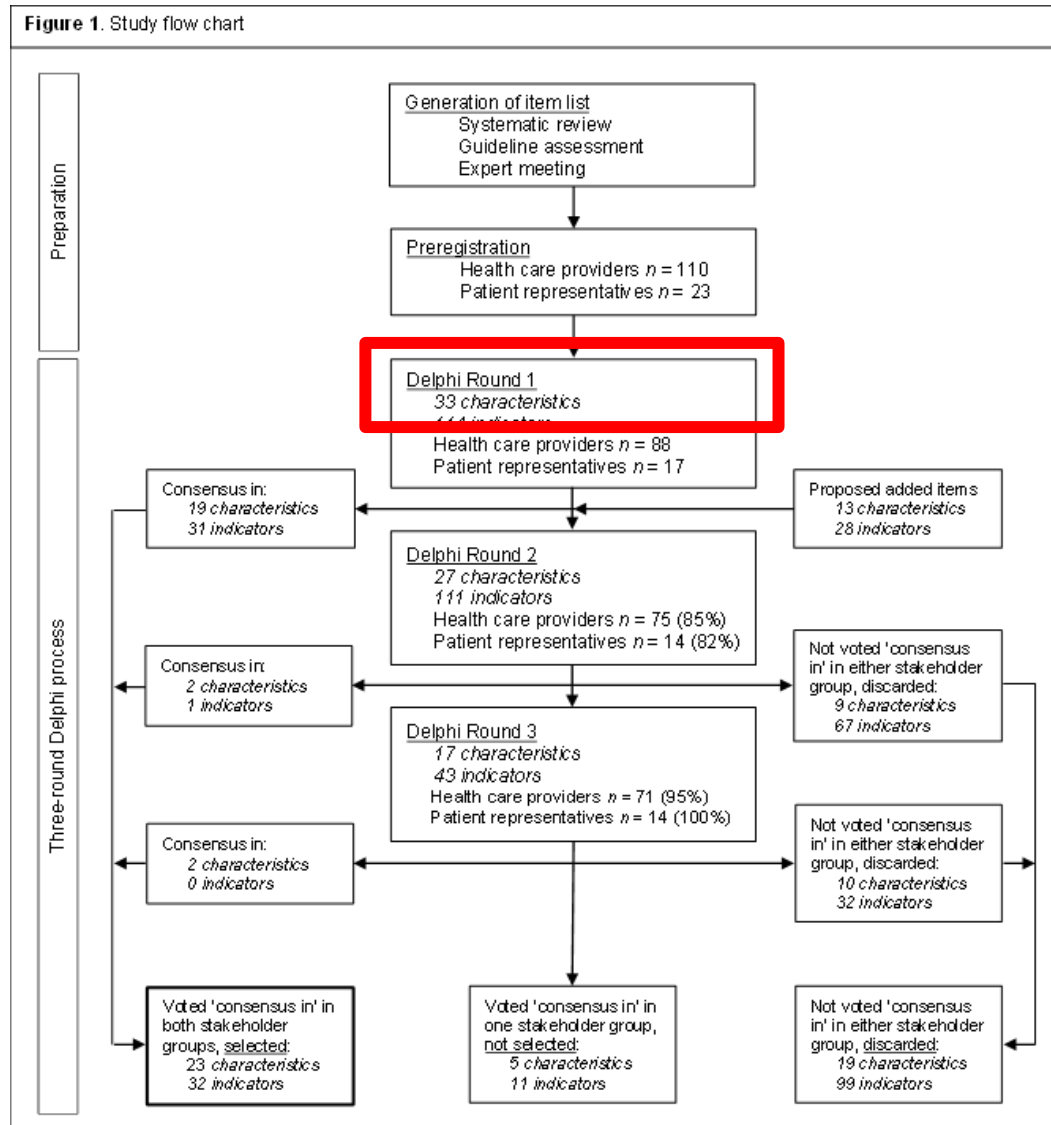
ERNs given money by EU to develop registries

**EPSA** European Pediatric Surgical Audit

Development of registry data structure

- core indicator set
- with patient representatives and healthcare professionals

# EA Registry Dataset revision



# Development of patient reported outcome measures

What if appropriate measurement instruments are not available?

e.g. Quality of life instruments

- Rather than use a generic tool such as PedsQL, develop condition specific tools



Health-related quality of life experiences among children and adolescents born with esophageal atresia: Development of a condition-specific questionnaire for pediatric patients



Michaela Dellenmark-Blom <sup>a,\*</sup>, John Eric Chaplin <sup>a</sup>, Vladimir Gatzinsky <sup>b</sup>, Linus Jönsson <sup>b</sup>, Helena Wigert <sup>c,d</sup>, Jeanette Apell <sup>b</sup>, Ulla Sillén <sup>b</sup>, Kate Abrahamsson <sup>a</sup>

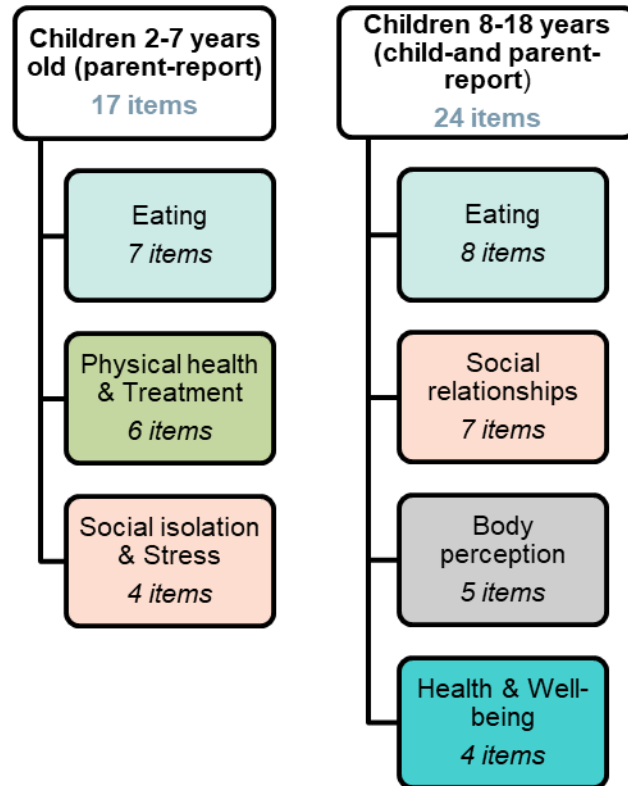
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# QoL



Cognitive debriefing  
of patients/caregivers  
with questionnaire  
items

Translated into English and being validated in UK, USA and South Africa



# Quality of Life

	Never	Rarely	Sometimes	Often	Always	Easy to understand?	Sensitive to answer?	Additional comments?/Improvement
Eating stresses my child	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/> Yes	<input type="checkbox"/> Yes	
						<input type="checkbox"/> No	<input type="checkbox"/> No	

# Limits to meaningful involvement

## Original article



**OPEN ACCESS**

## Development of a gastroschisis core outcome set

Benjamin Saul Raywood Allin,<sup>1</sup> Nigel J Hall,<sup>2</sup> Andrew R Ross,<sup>3</sup> Sean S Marven,<sup>4</sup> Jennifer J Kurinczuk,<sup>1</sup> Marian Knight,<sup>1</sup> on behalf of the NETS<sup>1G</sup> collaboration

Mothers of gastroschisis babies more likely to be:

- Young maternal age
- Drug, alcohol, tobacco users during conception and 1<sup>st</sup> trimester

Gastroschisis infants likely to be discharged from care and not having ongoing issues

Difficult group to reach, those that do engage likely to be those who have had poor experiences/outcomes

**We (Health Care Professionals and ePAGs) need to ensure that difficult to reach groups are not forgotten**

# Facebook Groups and other Social Media Forums



*"the scariest blue-spell happened some 10 days upon being home-  
...it was and still is the most horrifying moment of my entire  
life...this is one thing that stayed with me always and I still can  
feel the horror of it...from that moment on every feeding was a  
nightmare for me that I tried to cover up as hard as I could...but  
the PTSD [post traumatic stress response] with me stays..."*

# Qualitative research



## Parents' experiences of feeding children born with oesophageal atresia/tracheo-oesophageal fistula<sup>☆</sup>

Alexandra Stewart<sup>a,b,\*</sup>, Christina H. Smith<sup>a</sup>, Roganie Govender<sup>c,d</sup>, Simon Eaton<sup>e</sup>, Paolo De Coppi<sup>b,e</sup>, Jo Wray<sup>b</sup>

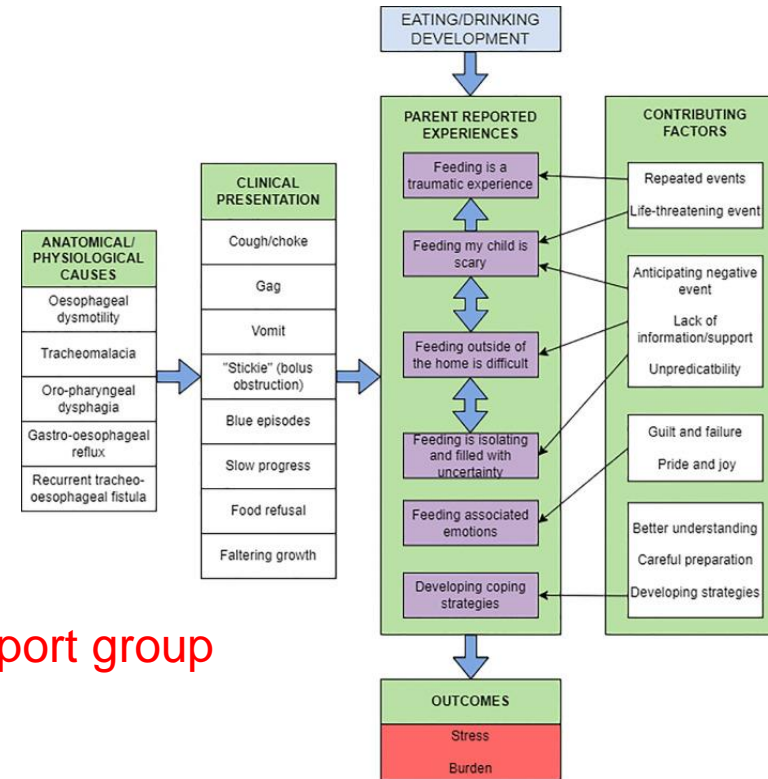
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<sup>d</sup>Head and Neck Academic Centre, University College London Hospital, 250 Euston Road, London, NW1 2PG, UK

<sup>e</sup>Stem Cells and Regenerative Medicine Section, University College London Institute of Child Health, 30 Guilford Street, London, WC1N 1EH, UK



- Specific forum moderated by patient support group
- Consent, ethics, disclosure

*"the scariest blue-spell happened some 10 days upon being home...it was and still is the most horrifying moment of my entire life...this is one thing that stayed with me always and I still can feel the horror of it...from that moment on every feeding was a nightmare for me that I tried to cover up as hard as I could...but the PTSD [post traumatic stress response] with me stays..."*

# How to engage

- Many mechanisms for engagement
- But.....be prepared for it to go more slowly than you want
- Better to have a well-designed co-production with appropriate approvals in place