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

Simon Eaton
Evidence-based medicine

Levels of Evidence

- Meta Analyses
- Randomised Controlled Trials
- Cohort Studies
- Case-Control Studies
- Case-Series or Case-Reports
- Editorials and Expert Opinion
Evidence-based medicine

Levels of Evidence

- Meta Analyses
- Randomised Controlled Trials
- Cohort Studies
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- Case-Series or Case-Reports
- Editorials and Expert Opinion

Common diseases (diabetes, stroke etc.)

Rare diseases

Whilst ensuring patients, parents and caregivers are at the centre
Esophageal atresia
2.4 / 10,000 live births
ERNICA Consensus Conference on the Management of Patients with Esophageal Atresia and Tracheoesophageal Fistula: Diagnostics, Preoperative, Operative, and Postoperative Management

Carmen Dingemann¹  Simon Eaton²  Gunnar Aksnes³  Pietro Bagolan⁴  Kate M. Cross⁵  Paolo Decoppi²,⁵  JoAnne Fruthof⁶  Piergiorgio Gamba⁷  Steffen Husby⁸  Antti Koivusalo⁹  Lars Rasmussen¹⁰  Rony Sfeir¹¹  Graham Slater¹²  Jan F. Svensson¹³  David C. Van der Zee¹⁴  Lucas M. Wessel¹⁵  Anke Widenmann-Grolig¹⁶  Rene Wijnen¹⁷  Benno M. Ure¹

Consensus meeting over 2 days
- 82 items voted on
- controversy on 21 items
ERNICA Consensus Conference on the Management of Patients with Esophageal Atresia and Tracheoesophageal Fistula: Diagnostics, Preoperative, Operative, and Postoperative Management

Carmen Dingemann, Simon Eaton, Gunnar Aksnes, Pietro Bagolan, Kate M. Cross, Paolo Decoppi, JoAnne Fruithof, Piergiorgio Gamba, Steffen Husby, Antti Koivusalo, Lars Rasmussen, Rony Sfeir, Graham Slater, Jan F. Svensson, David C. Van der Zee, Lucas M. Wessel, Anke Widenmann-Grolig, Rene Wijnen, Benno M. Ure

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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?
ERNICA Consensus Conference TEST

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

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?

- **9 - Fully Agree**: 15.0%
- **8**:
- **7**: 10.0%
- **6**: 5.0%
- **5**:
- **4**: 5.0%
- **3**:
- **2**:
- **1 - Strongly Disagree**: 25.0%

20 users voted
<table>
<thead>
<tr>
<th>Consensus yes/no</th>
<th>Agree/total votes</th>
</tr>
</thead>
<tbody>
<tr>
<td>10</td>
<td>The maximum number of esophageal dilatations for recurrent anastomotic strictures until a fundoplication should be considered is 5</td>
</tr>
<tr>
<td>11</td>
<td>Topical application of mitomycin C should be recommended as an option in patients with recurrent strictures</td>
</tr>
<tr>
<td>12</td>
<td>Intral esional/systemic steroids should be recommended as an option in patients with recurrent strictures</td>
</tr>
<tr>
<td>13</td>
<td>Customized stents /indwelling balloons should be recommended as an option in patients with recurrent strictures</td>
</tr>
<tr>
<td>14</td>
<td>24-hour-pH- or pH-impedance monitoring should be routinely used for monitoring children and adolescents with EA according a specific schedule</td>
</tr>
<tr>
<td>15</td>
<td>24-hour-pH- or pH-impedance monitoring should be routinely performed at time of discontinuation of antacid therapy</td>
</tr>
<tr>
<td>16</td>
<td>At least two additional pH studies should be routinely performed until transition</td>
</tr>
<tr>
<td>17</td>
<td>Endoscopies of the upper gastrointestinal tract should be routinely performed for monitoring children and adolescents with EA according a specific schedule</td>
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ERNICA Consensus Conference on the Management of Patients with Long-Gap Esophageal Atresia: Perioperative, Surgical, and Long-Term Management

Consensus meeting over 2 days
- 97 items voted on
- controversy on 19 items
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.
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
Keyhole surgery of premature neonates

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
QoL

- Children 2-7 years old (parent-report)
  - 17 items
  - Eating
    - 7 items
  - Physical health & Treatment
    - 6 items
  - Social isolation & Stress
    - 4 items

- Children 8-18 years (child-and parent-report)
  - 24 items
  - Eating
    - 8 items
  - Social relationships
    - 7 items
  - Body perception
    - 5 items
  - Health & Well-being
    - 4 items

Cognitive debriefing of patients/caregivers with questionnaire items

Translated into English and being validated in UK, USA and South Africa
<table>
<thead>
<tr>
<th>Eating stresses my child</th>
<th>Never</th>
<th>Rarely</th>
<th>Sometimes</th>
<th>Often</th>
<th>Always</th>
<th>Easy to understand?</th>
<th>Sensitive to answer?</th>
<th>Additional comments?/Improvement</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>☑</td>
<td>☑</td>
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<td>☑</td>
<td>☑ Yes</td>
<td>☑ Yes</td>
<td></td>
</tr>
</tbody>
</table>
Mothers of gastroschisis babies more likely to be:

- Young maternal age
- Drug, alcohol, tobacco users during conception and 1\textsuperscript{st} 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
“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...”
Parents’ experiences of feeding children born with oesophageal atresia/tracheo-oesophageal fistula

Alexandra Stewart1,2, Christina H. Smith1, Roganie Gowender1,2, Simon Eaton3, Paolo De Coppi4, Jo Wray5

1Department of Paediatrics and Paediatric Surgery, University College London, Charing Cross, 100 Fulham Road, London, SW3 6PT, UK
2Great Ormond Street Hospital for Children, Great Ormond Street, London, WC1N 3JH, UK
3Royal Berkshire Hospital, University College London Hospitals, Green Street, London, W6 9JU, UK
4Trinity and Maple Academic Centre, University College London Hospitals, 255 Euston Road, London, NW1 2PG, UK
5Sir John Premium and Children’s Hospital, University College London Hospitals, 255 Euston Road, London, NW1 2PG, UK

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

Eating/Drinking Development

ANATOMICAL PHYSIOLOGICAL CAUSES

• Oesophageal atresia
• Tracheo-oesophageal fistula
• Oesophago-tracheal dysplasia

CLINICAL PRESENTATION

• Gagging
• Vomiting
• “Bloat” (lack of distension)
• Blue episodes
• Failure to thrive
• Feeding difficulties

OUTCOMES

• Stress
• Burden

• Repeated events
• Life-threatening event
• Anticipating-negative event
• Lack of information/support
• Unpredictability

CONTRIBUTING FACTORS

• Guilt and failure
• Pride and joy

• Better understanding
• Careful preparation
• Developing strategies

“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 I 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