

## Research Award:

# Development and validation of age specific health related quality of life measures for patients with primary ciliary dyskinesia

**Awarded to:** Laura Behan and J. Lucas **Amount:** £9,000

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### Lay summary

Support from the AAIR Charity, co-funded by FP7 BESTCILIA, has led to age-specific quality of life measures for patients with primary ciliary dyskinesia (QOL-PCD). This work has provided the first disease-specific outcome measures to monitor PCD patients clinically, document the progression of their illness and assess the effectiveness of therapeutic interventions in clinical trials. Researchers from University of Southampton led the development and validation of QOL-PCD, involving patients from across Europe and North America. Our findings have shown QOL-PCD to be robust, reliable, responsive and valid.

In rare disease such as PCD, international collaborations are vital. It is important that QoL measures used in clinical trials are translated and tested comprehensively in each language. AAIR funding has enabled the QOL-PCD measures to be comprehensively translated in a number of languages for widespread use in international clinical trials across Europe, North America and the Middle East. The English, Danish, Dutch and German languages versions of the measures are already being used as outcome measures for a randomised clinical trial of azithromycin.

Finally, AAIR funding has enabled the PhD student working on this study to analyse the data, prepare a number of manuscripts, and submit her final thesis. It has also allowed for findings of the study to be presented to an international forum at the European Respiratory Society Congress, American Thoracic Society and at the COST Action BEAT-PCD Training School in Paris <http://www.beatpcd.org/>.

We are currently applying for external grants to support the development and validation of Parental Impact questionnaires, for carers of children with PCD under the age of 6 years.

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### Publications

- Behan L, Rubbo B, Lucas JS, Dunn Galvin A. The patient's experience of primary ciliary dyskinesia: a systematic review. Qual Life Res. 2017 Mar 30.

- Behan L, Leigh MW, Dell SD, Dunn Galvin A, Quittner AL, Lucas JS. Validation of a health-related quality of life instrument for primary ciliary dyskinesia (QOL-PCD). *Thorax*. 2017 Feb 28. pii: thoraxjnl-2016-209356.
  - Dell SD, Leigh MW, Lucas JS, Ferkol TW, Knowles MR, Alpern A, Behan L, Morris AM, Hogg C, DunnGalvin A, Quittner AL. Primary Ciliary Dyskinesia: First Health-related Quality-of-Life Measures for Pediatric Patients. *Ann Am Thorac Soc*. 2016 Oct;13(10):1726-1735.
  - Behan L, Dimitrov BD, Kuehni CE, Hogg C, Carroll M, Evans HJ, Goutaki M, Harris A, Packham S, Walker WT, Lucas JS. PICADAR: a diagnostic predictive tool for primary ciliary dyskinesia. *Eur Respir J*. 2016 Apr;47(4):1103-12.
  - Lucas JS, Behan L, Dunn Galvin A, Alpern A, Morris AM, Carroll MP, Knowles MR, Leigh MW, Quittner AL. A quality-of-life measure for adults with primary ciliary dyskinesia: QOL-PCD. *Eur Respir J*. 2015 Aug;46(2):375-83.
  - Behan L, Dunn Galvin A, Rubbo B, Masefield S, Copeland F, Manion M, Rindlisbacher B, Redfern B, Lucas JS. Diagnosing primary ciliary dyskinesia: an international patient perspective. *Eur Respir J*. 2016 Oct;48(4):1096-1107.
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## Presentations

- Laura Behan: Health related quality of life measures for PCD. Cost Action BEAT PCD Inaugural Conference 2016.
  - Jane Lucas: American Thoracic Society, San Francisco May 2016. Scientific symposium: The link between ciliary assembly defects, neonatal respiratory distress and bronchiectasis in adulthood: a primer on primary ciliary dyskinesia.
  - Jane Lucas: European Respiratory Society Congress, London September 2016. Scientific Symposium: Diagnosing primary ciliary dyskinesia (PCD) in a molecular age.
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## Posters

- Laura Behan, Alexandra L. Quittner, Anika Lofruchte, Anu Kalayamthanam, Aristoula Toliop Audrey DunnGalvin, Bridget Contreras, Christine Edelbusch, Claudia E. Kuehni, Claudius Werner, Elizabeth Maurer, Eric G. Haarman, Helene Elgaard Kobbarnagel, Irma Bon, Konstantinos Giannakou, Lea Heisch, Maria Philipsen, Niels Rutjes, Panagiotis Kouis, Tamara Paff, Jane S Lucas. Translation of QOL-PCD: a cross-cultural patient-reported outcome measure for patients with primary ciliary dyskinesia. European Respiratory Society Congress 2016.

- Claire O'Neill, Guillaume Thouvenin, Laura Behan, Delphine Habouria, Harriet Corvol, Annick Clément, Jane S Lucas, Aline Tamalet. French translation and linguistic validation of the QOL-PCD, a quality of life questionnaire for patients with Primary Ciliary Dyskinesia. European Respiratory Society Congress 2016.
  - Behan, Laura. Dunn Galvin, Audrey. Alpern, A. Morris, AM. Carroll, MP. Knowles, MR. Leigh, MW. Quittner, AL. Lucas, JS. Development, Translation and Validation of Health-Related Quality of Life Measures for Primary Ciliary Dyskinesia. Cost Action BEAT-PCD Training School 2016.
  - Behan L, Dunn Galvin A, Alpern A, Morris AM, Carroll MP, Knowles MR, Leigh MW, Quittner AL, Lucas JS. Development and validation of QOL-PCD – collaboration, participation and recruitment. Cost Action BEAT-PCD Training School 2016.
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