(https://www.uzleuven.be/polca). Liver volume was measured using CT-volumetry and adapted as height-adjusted total liver volume (htLV). Combined liver-kidney transplant patients (n = 9) were excluded.

Results: For 198 patients, serial POLCA scores were available. The study group consisted of young (54.8y ± 11.3), mostly female (83%) patients with predominant ADPKD (63%). Median time of follow-up was 48 months. Liver volumetry was available for 96 patients, showing a median htLV of 1967 ml.

There was a significant correlation (Spearman's rank) between POLCA severity of perceived illness (SPI) score and htLV (r = 0.48; 0.30–0.63). Patients who underwent LT had significantly higher scores on all POLCA subscales and htLV, compared to those not considered to be LT candidates (table 1). SPI score ≥16.5 predicted the need of LT with a sensitivity of 81.3% and a specificity of 88.9%.

Table: Comparison of POLCA subscales and htLV

POLCA	No LT (n = 171)	LT (n = 18)	*p* value
Severity of perceived illness	10.0 (6.0; 15.0)	23.5 (18; 26)	<0, 0001
POLCA	No LT (n = 122)	LT (n = 17)	
GERD complaints	2.0 (0.0; 5.0)	7.0 (4.5; 12.5)	<0, 0001
Impact on food intake	2.0 (0.0; 5.0)	6.0 (4.5; 9.0)	0, 0004
Perception of enlarged LV	6.0 (3.0; 8.0)	10.0 (7.5; 12.0)	<0, 0001
	No LT (n = 71)	LT (n = 16)	
htLV (ml)	1707 (1173; 2690)	3607 (2901; 4337)	<0, 0001

Longitudinal data showed a significant correlation between the change in SPI score and the change in htLV (r = 0.45; 0.26–0.61) and a significant reduction in htLV (−80 ml) by SA resulted in a decrease in SPI score (−6.0 vs +4.5).

Conclusion: This prospective study confirms the use of the POLCA score as a self-report instrument to assess the severity of PCLD-related symptoms. It is a clear reflection of both evolving symptom severity as well as objective changes in liver volume. Our findings highlight the potential of the POLCA score as a tool for longitudinal follow-up of PCLD patients and as a guide for clinicians when evaluating the need for medical or surgical intervention.

FRI253

Improvements in quality of life in odevixibat responders and nonresponders: an analysis of pooled data from the PEDFIC 1 and PEDFIC 2 studies

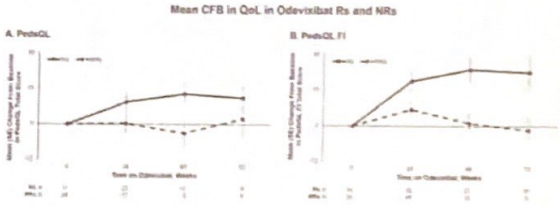
Cara L. Mack[1], Chad Gwaltney[2], Quanhong Ni[3], Qifeng Yu[3], Velichka Valcheva[3], Lise Kjems[3], Patrick Horn[3]. [1]*Children's Hospital Colorado, University of Colorado School of Medicine, Aurora, CO, United States;* [2]*Gwaltney Consulting, Westerly, RI, United States;* [3]*Albireo Pharma, Boston, MA, United States*

Email: cara.mack@childrenscolorado.org

Background and aims: Patients with progressive familial intrahepatic cholestasis (PFIC) may have debilitating pruritus that can impact their and their families' quality of life (QoL). The phase 3 PEDFIC 1 and PEDFIC 2 studies evaluated the efficacy and safety of odevixibat, an ileal bile acid transporter inhibitor, in patients with PFIC. In these studies, odevixibat improved serum bile acids (sBAs), pruritus, and sleep; QoL was also evaluated as an exploratory endpoint. Here, using pooled data from these studies, we describe patient- and family-focused QoL changes in odevixibat responders (Rs) and nonresponders (NRs).

Method: In PEDFIC 1, children with PFIC received placebo or odevixibat (40 or 120 µg/kg/day) for 24 weeks. PEDFIC 2 is an ongoing 72–week extension study that enrolled patients from PEDFIC 1 or new patients; all patients in PEDFIC 2 receive odevixibat 120 µg/kg/day. Data from these studies were pooled from patients' first dose of odevixibat to a cut-off date of 4 December 2020. To assess QoL, caregivers of patients ≥2 years old completed the Pediatric QoL Inventory (PedsQL) and family impact (FI) questionnaires, which output total impact scores ranging from 0 to 100; higher scores indicate greater QoL. Treatment R was defined as sBA reductions of ≥70% or levels ≤70 µmol/L and/or ≥1-point drop from baseline in caregiver-reported pruritus score (on 0–4 scale) at last available assessment.

Results: In the pooled population, 49/84 (58%) met R criteria, and 35/84 (42%) were NRs. In Rs, mean age was 4.8 years and 57% were female; in NRs, mean age was 5.5 years and 37% were female. Among patients with available QoL data (PedsQL: R: n = 31, NR: n = 22; FI: R: n = 46, NR: n = 33), mean (SE) PedsQL and FI total scores at baseline indicated that both Rs (PedsQL: 56.6 [2.3]; FI: 49.9 [2.8]) and NRs (PedsQL: 55.0 [3.5]; FI: 48.9 [3.4]) had impaired QoL at least some of the time, on average. At week 72 in patients with available data, the mean (SE) change from baseline (CFB) in PedsQL total score was 11.0 (4.6) in Rs and 2.3 (8.6) in NRs; the mean (SE) CFB in PedsQL FI total score was 22.5 (7.0) in Rs and −1.7 (4.6) in NRs (Figure).



Conclusion: Odevixibat Rs and, particularly, their families, experienced QoL improvements that were sustained over time; QoL for NRs and their families was largely unchanged with up to 72 weeks of treatment. Further analysis of specific domains within PedsQL and FI that were most highly impacted in Rs is ongoing.

FRI254

Total, primary, and secondary serum bile acid changes and pruritus improvement during odevixibat treatment in patients with progressive familial intrahepatic cholestasis

Henkjan J. Verkade[1], Folkert Kuipers[1], Quanhong Ni[2], Velichka Valcheva[2]. [1]*Department of Paediatrics, University of Groningen, Beatrix Children's Hospital/University Medical Centre Groningen, Groningen, Netherlands;* [2]*Albireo Pharma, Inc., Boston, MA, United States*

Email: h.j.verkade@umcg.nl

Background and aims: Children with progressive familial intrahepatic cholestasis who received odevixibat in the 24-week PEDFIC 1 study had significant reductions vs placebo-treated patients in total serum bile acids (sBAs) and pruritus. Here, we evaluated changes in sBAs and pruritus in patients from PEDFIC 1 categorised by sBA response (R) level and by factoring in ursodeoxycholic acid (UDCA) use.

Method: Patients eligible for PEDFIC 1 had elevated sBAs and significant pruritus at screening. Concomitant UDCA was allowed provided the patient's dose was stable. Three categories of patients among those randomised to odevixibat (n = 42) were analysed here: sBA Rs (ie, sBAs reduced ≥70% from baseline [BL] or levels ≤70 µmol/L), sBA partial Rs (PRs [ie, did not meet sBA R criteria but had sBAs reduced ≥30%), and sBA nonresponders (NRs [ie, did not meet either sBA R or PR criteria). Parameters evaluated included sBA composition (ie, total, primary, and secondary BAs, with UDCA concentration included as secondary BA) as measured by liquid chromatography–tandem mass spectrometry and pruritus as rated by caregivers (range: 0–4; higher scores indicate worse symptoms, with pruritus R defined as a ≥1-point reduction from BL); pruritus outcomes were also compared by whether patients had concomitant UDCA use and/or sBA R.

	Non-augmented n = 259	Augmented n = 315	p value (multivariable) *
Age (years)	50 ± 15	58 ± 10	<0.0001*
Women (%)	48.3	43.8	0.29*
BMI (kg/m^2)	24.4 ± 3.9	24.8 ± 4.5	0.96*
FEV1 (%)	78 ± 26	47 ± 18	<0.0001*
CAT (points)	12 ± 8	19 ± 7	<0.0001*
LSM (kPa)	6.5 ± 5.5	6.1 ± 3.9	0.042
LSM ≥7.1 kPa (%)	22.0	19.4	0.020
ALT (% ULN)	76 ± 45	77 ± 42	0.89
AST (% ULN)	74 ± 28	69 ± 26	<0.0001
GGT (% ULN)	92 ± 110	86 ± 74	0.021

Abbreviations: BMI, body mass index; FEV1, forced expiratory volume in 1 second; CAT, COPD assessment test; LSM, liver stiffness measurement; ALT, alanine aminotransferase; AST, aspartate aminotransferase; GGT, gamma-glutamyltransferase; ULN, upper limit of normal. *Univariate.

Conclusion: AAT augmentation therapy seems to have a beneficial association to the liver phenotype of Pi*ZZ individuals. Prospective studies are needed to confirm this observation.

FRI251

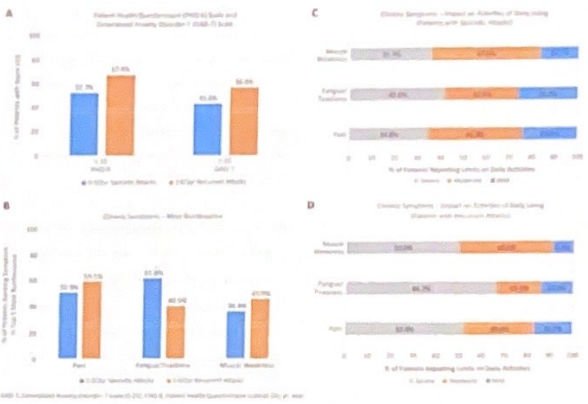
Impact of acute hepatic porphyria attack frequency on patient-reported outcomes: results from the porphyria worldwide patient experience research (POWER) study

Amy Dickey[1], Kristen Wheeden[2], Sue Burrell[3], Rocco Falchetto[4], Jasmin Barman-Aksözen[4,5], Alison Bulkley[6], Stephen Meninger[7], Stephen Lombardelli[8], Danielle Nance[9]. [1]*Massachusetts General Hospital, Boston, United States;* [2]*American Porphyria Foundation, Bethesda, United States;* [3]*Global Porphyria Advocacy Coalition, Durham City, United Kingdom;* [4]*Swiss Society for Porphyria, Zurich, Switzerland;* [5]*Stadtspital Waid and Triemli, Institute of Laboratory Medicine, Zurich, Switzerland;* [6]*Kantar Health, New York, United States;* [7]*Alnylam Pharmaceuticals, Cambridge, United States;* [8]*Alnylam Pharmaceuticals, Maidenhead, United Kingdom;* [9]*Banner Health, Gilbert, United States*
Email: adickey@mgh.harvard.edu

Background and aims: Acute hepatic porphyria (AHP), a group of rare genetic diseases of haem biosynthesis, is characterised by neurovisceral pain attacks. This study evaluated the impact of AHP on patient-reported outcomes (PROs) and disease burden in AHP patients who experience sporadic or recurrent attacks.

Method: Adult patients having >1 AHP attack within the past 2 years or receiving intravenous hemin and/or glucose for attack prevention were recruited from the United States, Italy, Spain, Australia, Mexico, and Brazil and administered an online survey from January 19 to April 26, 2021. Patients taking givosiran were excluded. Descriptive and bivariate analyses were performed to evaluate differences between patients with sporadic attacks (annualised attack rate [AAR], <6 within past 2 years) and recurrent attacks (AAR, ≥6). Attacks included those leading to a hospitalisation, an emergency room visit, an outpatient doctor visit, or self-management. PROs were assessed with the Generalized Anxiety Disorder-7 (GAD-7) scale (0–21) and the Patient Health Questionnaire (PHQ-8) scale (0–24). Burden of chronic symptoms was also reported.

Results: Of the 92 AHP patients who completed the survey, 55 (60%; mean age, 40.3 years) reported sporadic attacks and 37 (40%; mean age, 42.3 years) reported recurrent attacks. Most patients were female (sporadic, 92.7%; recurrent, 86.5%), and the most frequent diagnosis was acute intermittent porphyria (sporadic, 83.6%; recurrent, 59.4%). A majority of patients in the sporadic (52.7%) and recurrent (67.6%) attack groups reported a PHQ-8 score ≥10, indicating moderate to severe depression; 43.6% and 56.8% of patients in the sporadic and recurrent groups, respectively, reported a GAD-7 score ≥10, indicating moderate to severe anxiety (Figure 1A). Pain was reported as one of the top 3 most burdensome chronic symptoms in the sporadic (50.9%) and recurrent (59.5%) groups (Figure 1B). Of patients reporting their daily activities being limited by severe chronic symptoms, 83.3% of those in the sporadic (Figure 1C) and 90.6% in the recurrent group (Figure 1D) reported muscle weakness as a top 3 symptom having a moderate to severe impact.



Conclusion: While disease burden appeared greater for AHP patients experiencing recurrent attacks, both sporadic and recurrent group patients experienced a substantial impact on physical, mental, and emotional quality of life.

FRI252

Large-scale, multi-centric prospective validation of the polycystic liver disease complaint-specific assessment (POLCA)

Antoon Billiet[1], Frederik Temmerman[1], Walter Coudyzer[2], Natalie Van den Ende[1], Isabelle Colle[3], Sven Francque[4], Ho Thien Anh[5], Stéphane De Maeght[6], Filip Janssens[7], Hans Orlent[8], Dirk Sprengers[9], Jean Delwaide[10], Sofie Decock[11], Jochen Decaestecker[12], Schalk van der Merwe[1], Jef Verbeek[1], Frederik Nevens[1]. [1]*University Hospitals KU Leuven, Gastroenterology and Hepatology, Leuven, Belgium;* [2]*University Hospitals KU Leuven, Radiology, Leuven, Belgium;* [3]*Algemeen Stedelijk Ziekenhuis Aalst, Gastroenterology and Hepatology, Aalst, Belgium;* [4]*Antwerp University Hospital, Gastroenterology and Hepatology, Antwerpen, Belgium;* [5]*Université Catholique de Louvain, Nefrology, Brussels, Belgium;* [6]*Grand Hôpital de Charleroi, Gastroenterology and Hepatology, Charleroi, Belgium;* [7]*Jessa Ziekenhuis, Gastroenterology and Hepatology, Hasselt, Belgium;* [8]*AZ Sint Jan Brugge, Gastroenterology and Hepatology, Brugge, Belgium;* [9]*GZA Antwerp, Gastroenterology and Hepatology, Antwerpen, Belgium;* [10]*C.H.U. de Liège, Gastroenterology and Hepatology, Liege, Belgium;* [11]*AZ Sint Lucas Brugge, Gastroenterology and Hepatology, Brugge, Belgium;* [12]*AZ Delta, Gastroenterology and Hepatology, Roeselare, Belgium*
Email: antoon_billiet@hotmail.com

Background and aims: Polycystic liver disease (PCLD) can lead to extensive hepatomegaly, often associated with severe complaints. Indication for somatostatin-analogues (SA) or liver transplantation (LT) is in part based on subjective, patient-reported symptoms. In 2014 the PCLD-complaint-specific assessment (POLCA) score was developed as a self-report instrument to objectively capture the presence and severity of disease-specific complaints (Temmerman F, J Hepatol 2014).

The aim of this study was to validate the POLCA score and investigate the correlation with liver volume and need for volume-reduction therapy.

Method: A five year prospective multi-centric study in 21 hospitals in Belgium gathered a cohort of 266 PCLD patients. Sequential data including POLCA score, liver volumetry and the need for volume-reduction therapy were recorded. Participants were asked to complete the POLCA questionnaire, as available online