


QUO VADIS

A movie poster for the film 'Quo Vadis'. The title 'QUO VADIS' is written in large, golden, serif letters at the top. Below the title, a man with dark hair, wearing Roman-style armor and a red cape, is shown in profile, shouting with his mouth wide open. The background is a dark, smoky or dusty environment.

Quo Vadis Biliary Atresia

A European Perspective

Ekkehard Sturm , Christoph Slavetinsky, Claus Petersen

Focus on improving native liver survival in biliary atresia

1. Screening
2. Prognostic markers
3. Adjuvant therapy



ORIGINAL ARTICLE

Hepatology

Awareness, referral and age at Kasai surgery for biliary atresia in Europe: A survey of the Quality-of-Care Task Force of ESPGHAN

**Florence Lacaille¹ | Emanuele Nicastro² | Piotr Czubkowski³ |
Cristina Campos Gonçalves⁴ | Thu Giang Le Thi⁵ | Sibylle Koletzko^{5,6} | the
ESPGHAN Quality-of-Care Task Force**

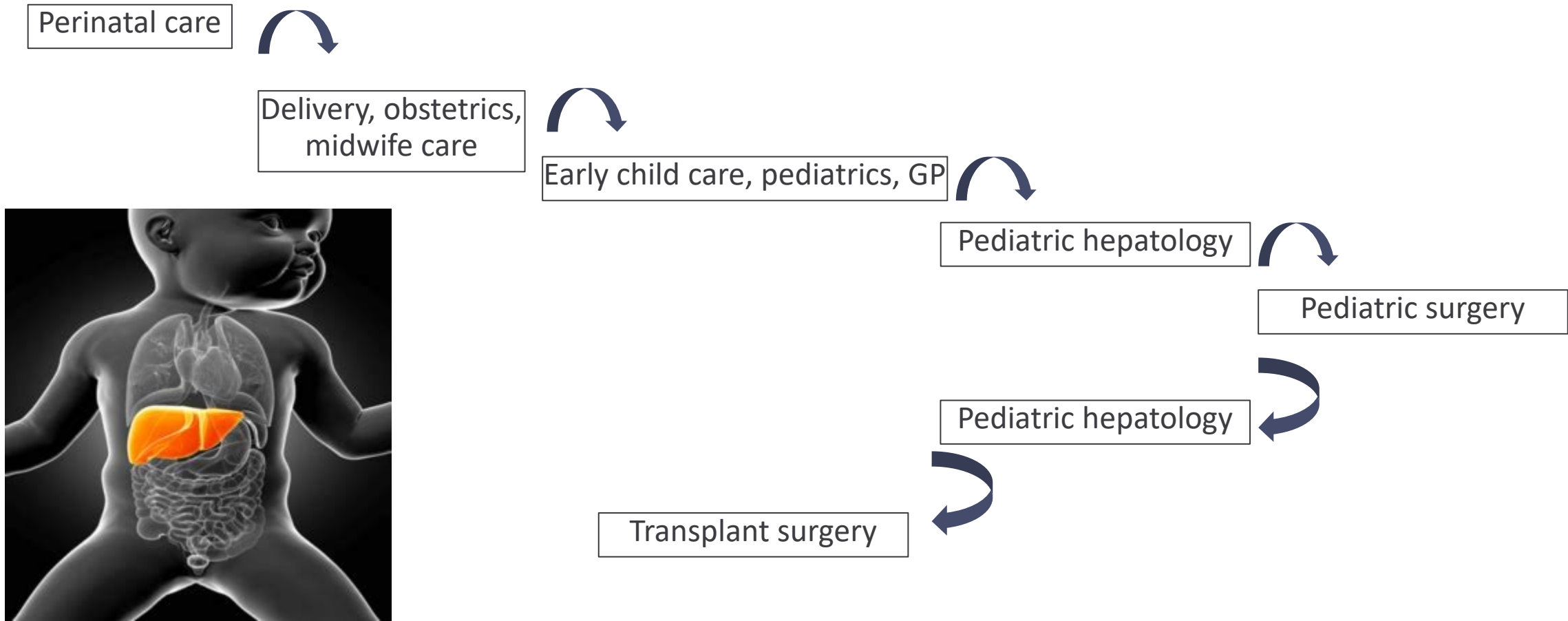
INVITED COMMENTARY

Old habits die hard: The age at Kasai portoenterostomy in European infants with biliary atresia

Willem S. Lexmond | Henkjan J. Verkade 



Complex and variable patient pathway in biliary atresia



Therapeutic trials for Biliary Atresia

TABLE 1 Selected ongoing clinical trials investigating repurposed therapeutics for biliary atresia.

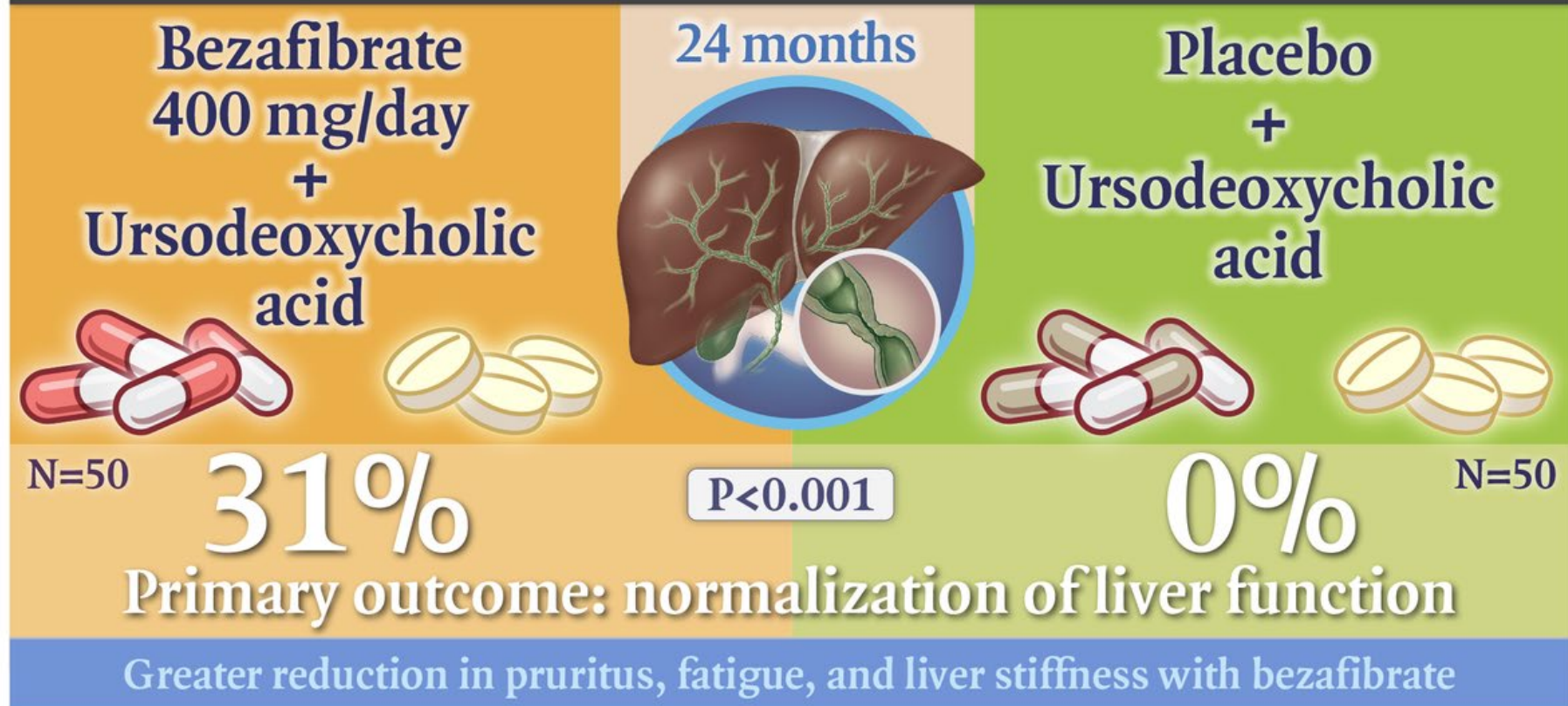
Drug	Identifier (s)	Phase	Design	Locations	Target Size	Status
Obeticholic Acid	NCT05321524	Phase II	Non-randomized, sequential assignment, open-label	>10 countries	32	Not Yet Recruiting
Odevixibat	NCT04336722	Phase III	Randomized, double-blind, placebo-controlled	>10 countries	200	Recruiting
Bezafibrate	JPRN-jRCTs031210066	Phase II	Non-randomized, single-arm, open-label	Japan (single site)	10	Recruiting
Maralixibat	NCT04524390; EUCTR2020-000974-22-GB	Phase II	Randomized, double-blind, placebo-controlled	USA, France, Canada, Poland, Germany, Italy, United Kingdom	72	Recruiting
Rituximab	ChiCTR2000031738	Phase IV	Non-randomized, controlled	China (single site)	6	Recruiting
Granulocyte-colony stimulating factor	NCT04373941	Phase II	Randomized, controlled, open-label	Pakistan, Vietnam	200	Recruiting
Mitomycin C	CTRI/2018/12/016495	Phase II	Randomized, controlled, open-label	India	50	Not Yet Recruiting



Bezafibrate in biliary atresia?

Bezafibrate in Primary Biliary Cholangitis

MULTICENTER, RANDOMIZED, DOUBLE-BLIND PHASE 3 TRIAL

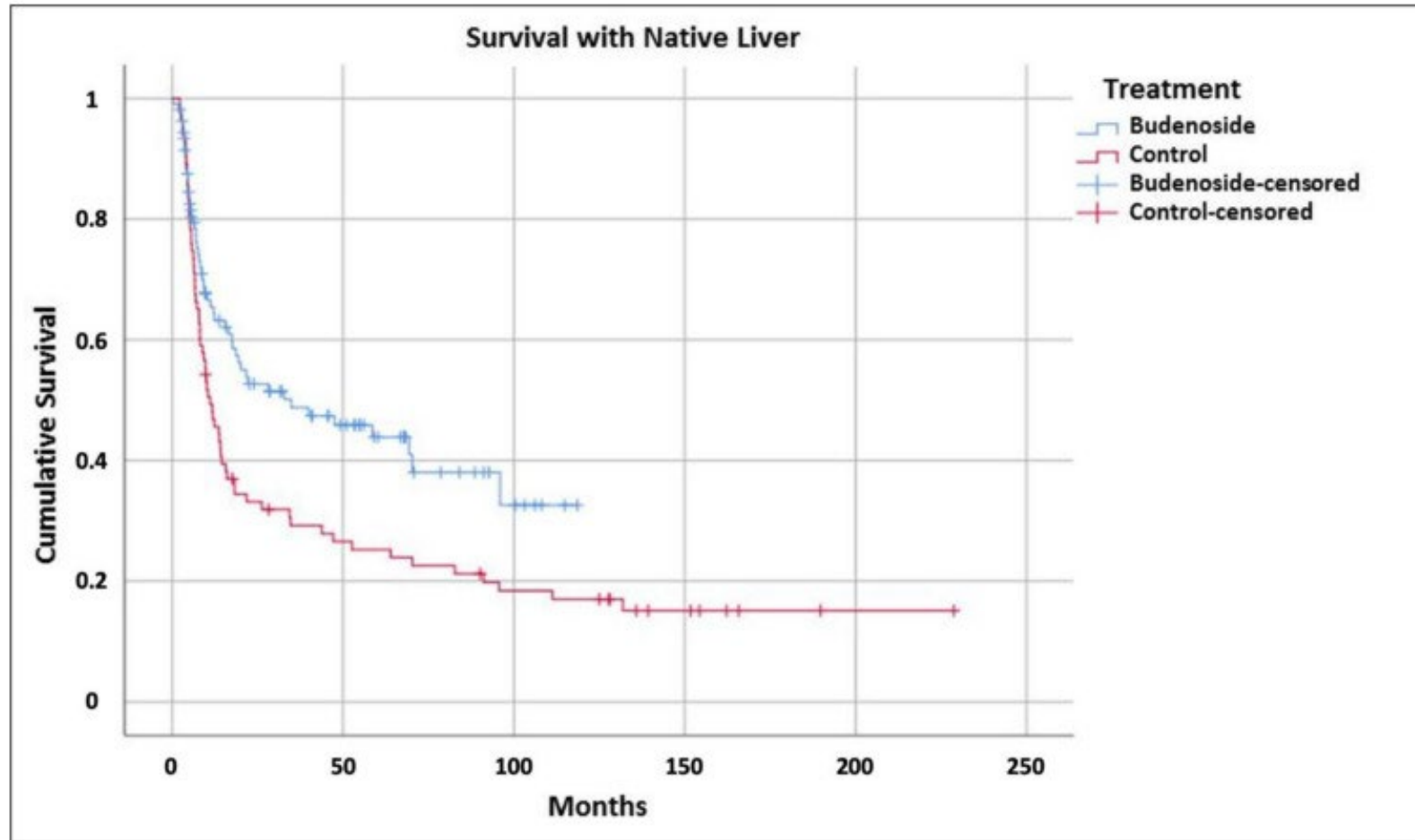


The NEW ENGLAND JOURNAL of MEDICINE

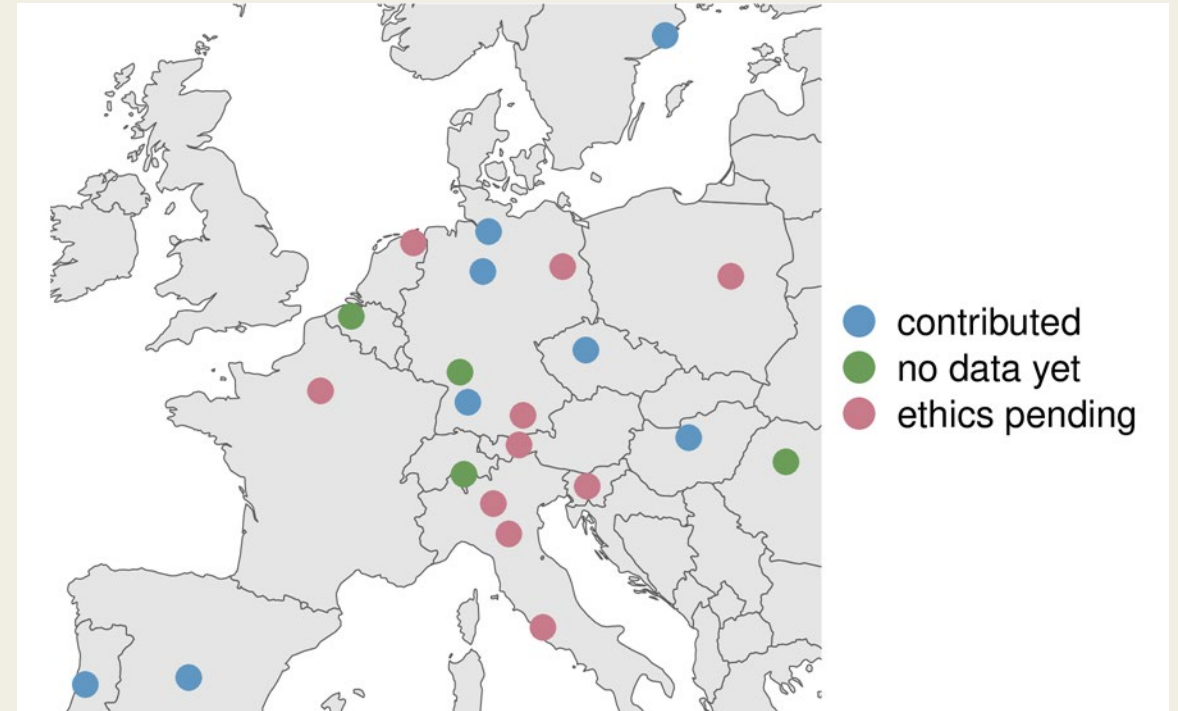
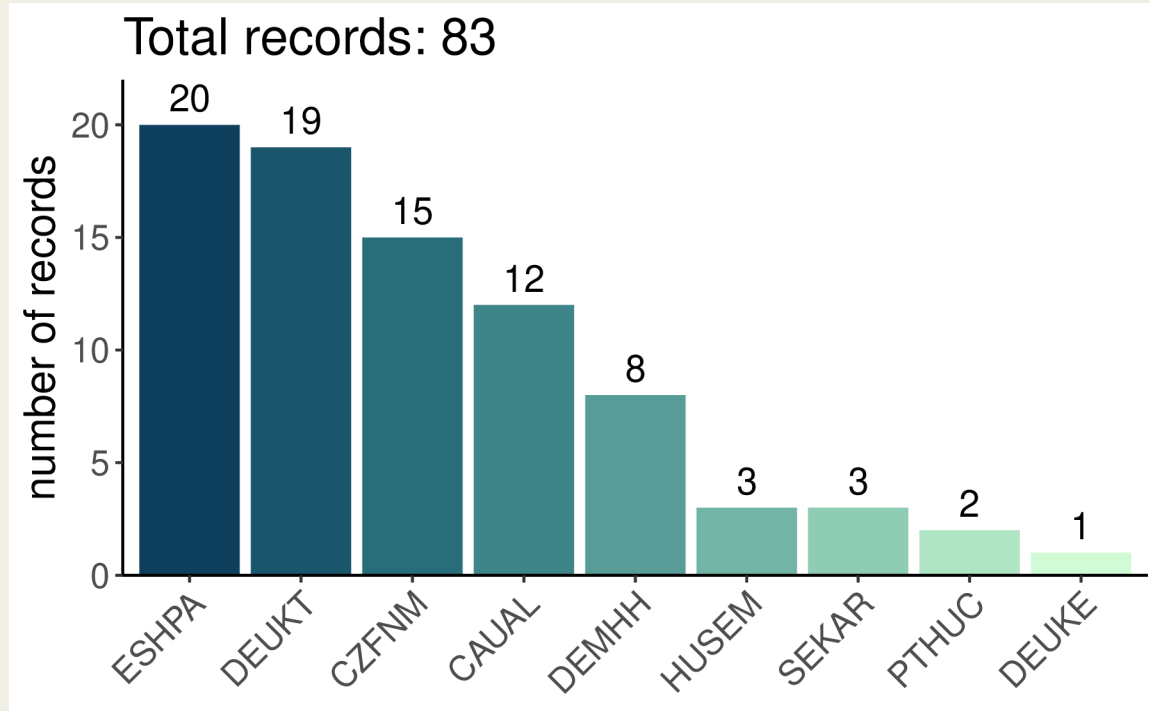
Corpechot et al. 2018



Adjuvant Therapy with Budesonide Post-Kasai Reduces the Need for Liver Transplantation in Biliary Atresia



One year of EBAR – Where are we?



Quo Vadis Biliary Atresia- Summary and Perspective

1. Combine biomarkers and screening tools?
2. Investigator Initiated Trials with novel compounds?
3. Use available registries to collect data and expand
4. Establish strategy to acquire funding



BARE 

BILIARY ATRESIA RESEARCH & EDUCATION

Jennifer Lau | President, Co-Founder

About BARE

Founded in 2022, BARE strives to facilitate connections within our community and bring all stakeholders together to drive research and education for better outcomes in diagnosis, treatment and management within the Biliary Atresia community.



www.bareinc.org



BARE 2024 Symposium

Hot Topics - Scientific Day

- Pathogenesis of BA
- MMP7 as a Biomarker
- Kasai vs Primary Transplant
- Biliary Atresia Microbiome
- Clinical Challenges in BA
- Early Detection
- Newborn Screening



BARE 2024 Symposium

Hot Topics - Patient & Family Day

- Lack of Educational Resources
 - Access to Care - Insurance Barriers
 - More Tools for Pediatricians & OBGYN
 - Patient and Family Support
 - Early Detection
 - Newborn Screening
-



Community Pain Point

Early Detection

When discussing with other families about their journey with Biliary Atresia, the common pain point was lack of early detection with the disease. Although a patient's BA journey can be very different from one patient to another, the beginning is similarly challenging.

The lack of newborn screening policy across all medical systems is detrimental to outcomes in diagnosis and treatment.

Late detection leads to significant morbidity and mortality.





Biliary Atresia

Newborn Screening

On September 8, 2023, BARE submitted a nomination package to the Advisory Committee on Heritable Disorders in Newborns and Children for Biliary Atresia to be added to the Recommended Uniform Screening Panel for newborns.

This committee operates within the US Department of Health and Human Services.



Why NBS?

Treatment is available

If no treatment by 60 days of life, liver failure and significant morbidity/ mortality set in ; when treatment is given early, transplant can be delayed

Transplant is not a cure: no split liver policy in the US, limited living donors

NBS can help solve these issues through access to early treatment leading to life-saving therapy and a chance to thrive with their native liver for longer

NBS is simple and accurate.



Tools To Help

Biliscreen.org

BiliScreen

This tool evaluates levels of direct or conjugated bilirubin, to quickly identify infants with biliary atresia
(please visit bilitool.org to evaluate levels of total bilirubin)

Creating SpACE

Speciality Access for Children Everywhere



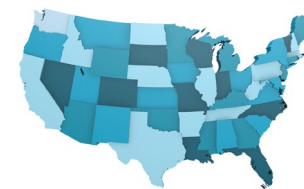
BARE & NBS:

What's Next For The Community



Task Force

Establish a diverse panel of stakeholders to advise, conduct and lead in policy, clinical and advocacy efforts for the BARE newborn screening initiatives.



State NBS

In collaboration with providers and BARE patient advocates, the NBS Task Force will lead policy efforts to add BA to state newborn screening panels.



Federal RUSP

BARE will continue to advocate our submission of the RUSP application to HRSA to include BA at the federal level for NBS

Thank You

Contact Us:

newbornscreening@bareinc.org

