

## Researcher Spotlight

# A LEADER IN TREATING PEDIATRIC RARE DISEASES IN VIETNAM

**Dr. Anh-Hoa  
Pham Nguyen**

In 2021, Cures Within Reach funded **Dr. Anh-Hoa Pham Nguyen** at the **Vietnam National Children's Hospital** to support her multi-site Phase IIB trial testing Granulocyte-Colony Stimulating Factor, a biologic medicine, to treat children with Biliary atresia (BA). BA is a rare congenital liver disease that causes bile duct inflammation and destruction, with a higher disease burden in low and lower-middle income countries.

Dr. Nguyen has been a pediatrician at Vietnam National Hospital of Pediatrics (NHP) for the past 20 years, and the head of NHP's Hepatology department since 2013. Her research focus is on children's hepatic diseases. She received Young Investigator Awards in both Japan and Taiwan. Dr. Nguyen credits her perseverance, concentration and honesty for motivating her mission to impact children's lives.

### Profile

Dr. Anh-Hoa Pham Nguyen earned her medical degree and PhD at Hanoi Medical University in Pediatric Gastroenterology.

### CWR Funding



This **Phase IIB clinical trial** is investigating whether the administration of Granulocyte-Colony Stimulating Factor to BA patients following surgical treatment improves outcomes. Dr. Nguyen and this trial represent three of Cures Within Reach's communities:

- **Rare diseases**
- **Health Equity**
- **LMICs**



Cures Within Reach is testing already approved treatments for unsolved diseases:  
driving more treatments to more patients more quickly

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