



**AN OBSERVATIONAL, NON-  
INTERVENTIONAL, MULTI-CENTER, MULTI-  
NATIONAL STUDY OF PATIENTS WITH  
ATYPICAL HEMOLYTIC-UREMIC SYNDROME  
(aHUS REGISTRY)**



# aHUS Registry Overview

The aHUS Registry is an observational, non-interventional, multinational study that will collect important data on the progression of an ultra-rare disease, aHUS, regardless of therapy.

The aHUS Registry has been developed in order to describe the real world outcomes of aHUS, collecting data on a wide range of patients from all over the world



# aHUS Registry Objectives

- To establish a robust, global database of patients with aHUS
- To characterize the clinical course of patients with aHUS
  - Including long-term consequences of TMA complications/manifestations, other morbidities, and mortality
- To enhance the understanding of aHUS by publishing analyses of Registry data



## Physician Benefits

- Contribute to one global, longitudinal database of patients with aHUS
- Collaborate with other physicians from around the globe dedicated to aHUS
- Access data and receive regular feedback with Registry updates and findings
- Increase knowledge about treatment outcomes in patients with aHUS



# Benefits to Patients

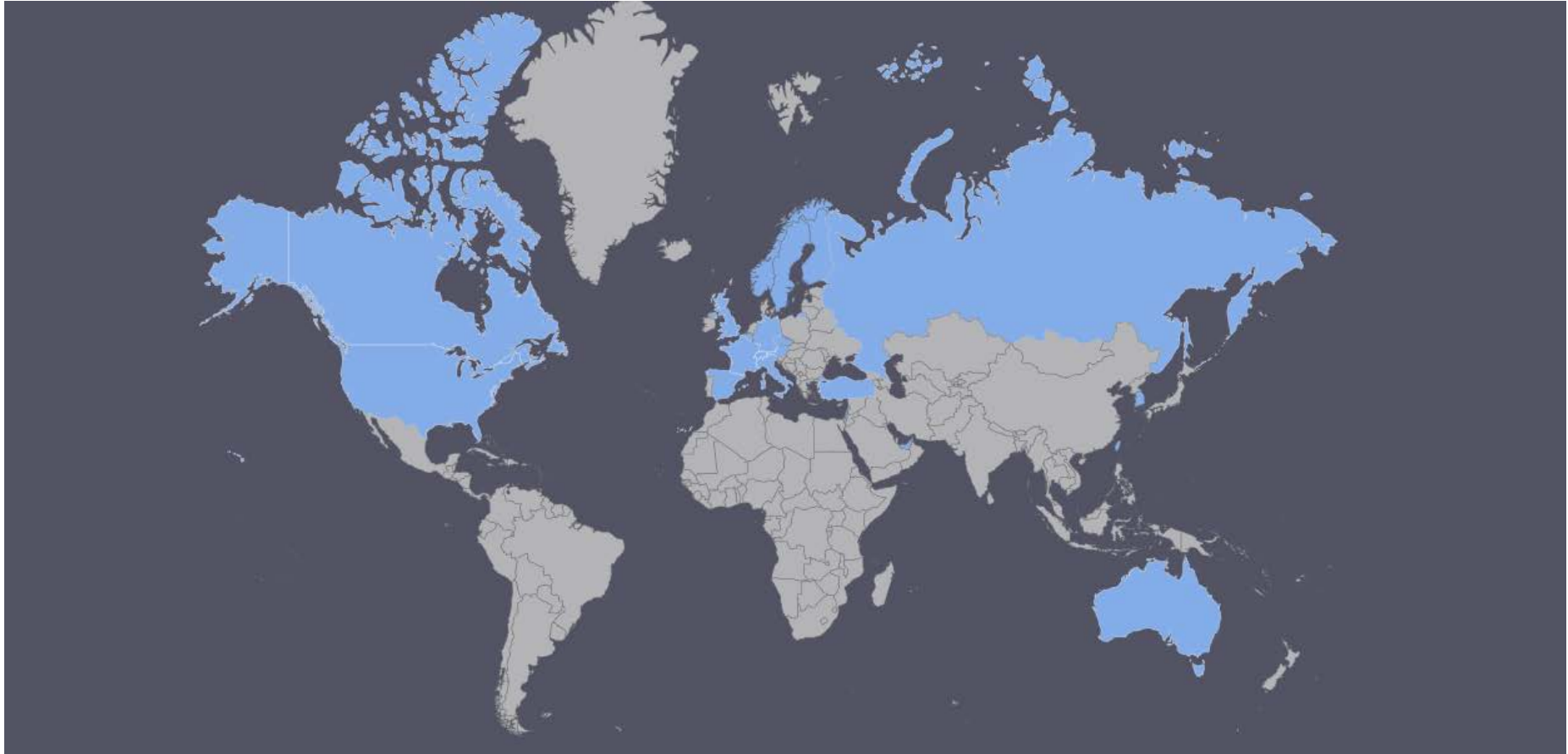
- Personal contribution of data that can be used to help improve the medical understanding, knowledge and management of aHUS
  - Data collected can provide benefits to other patients with aHUS
- Participating patients have the potential for ready access to new information about aHUS that may be of interest to them or their families
- Information about Registry study and aHUS
  - Evaluating options for distributing updates to patients; e.g., patient newsletters
  - General information may be published on Registry website



# Patient & Physician Participation

- All Physicians managing patients with aHUS are eligible to participate
- All patients of any age diagnosed with aHUS, regardless of treatment;
- Diagnosis defined by:
  - Clinical diagnosis of aHUS
  - Patients with or without an identified complement regulatory factor genetic mutation or anti-complement factor antibody
  - ADAMTS13 >5% (if performed)
- Exclusion criteria: patients with Hemolytic Uremic Syndrome (HUS) only due to Shiga Toxin

# Global Presence



The Goal is for the aHUS Registry to become the largest, most comprehensive database on aHUS

- First patient enrolled in April 2012
- As of August 2018
  - 351 sites across 23 countries were initiated
  - 1770 patients enrolled

Global aHUS Registry



# aHUS Registry Data Collection

- Physician-Reported Data
  - Data will be collected at study enrollment and every 6 months thereafter for all patients in the study
  - Data entry includes information on demographics, medical and disease history, TMA manifestations, aHUS diagnosis, symptomology, clinical outcomes, concomitant medications, and safety. Pregnancy and lactation data will also be collected (if applicable)
  - All necessary information will be gathered from patient medical records and will be entered via a secure web portal and maintained anonymously





# aHUS Registry Data Collection

- Patient Reported Outcomes
  - Patients complete questionnaires at study enrollment and every six months thereafter
    - Paper copies completed by patient
  - Patient Reported Outcomes include:
    - Patient Reported aHUS Symptoms
    - Patient Questionnaire
      - Resource Utilization
      - General Health
      - Work Status
    - FACIT-Fatigue

As of August 2018, the membership includes:

Name	Country	Institution
Christoph Licht	Canada (Chair)	The Hospital for Sick Children and University of Toronto
Gema Ariceta	Spain	Hospital Valle d'Hebron, Barcelona
Gianluigi Ardissino	Italy	Fondazione IRCCS Cá Granda, Ospedale Maggiore Policlinico, Milan
David Cohen	US	Columbia University, New York
Fadi Fakhouri	France	CHU de Nantes-Hôpital Hotel Dieu, Nantes
Véronique Frémeaux-Bacchi	France	Hôpital Européen Georges Pompidou, Paris
Larry Greenbaum	US	Emory University, Atlanta
Nicole Isbel	Australia	Princess Alexandra Hospital, Brisbane
Sally Johnson	UK	Newcastle Upon Tyne Hospital, Newcastle
Franz Schaefer	Germany	University Clinic Pediatric Nephrology, Heidelberg
Marie Ann Scully	UK	University College London Hospitals NHS Foundation Trust, London
Johan Vande Walle	Belgium	UZ Gent Dienst Nefrologie, Gent
Leonard Woodward	UK	aHUS Alliance (Patient Advocate)
Benjamin Miller	US	Alexion Pharmaceuticals, Inc.

- Global nature of the aHUS Registry will enable ability to describe global trends and/or differences across regions
- Longevity of the aHUS Registry will allow for true understanding of long term outcomes of disease and treatment
- Creation of one global database will eventually yield a denominator large enough for complex longitudinal and statistical analyses

# Contact Details

- If you would like more information, please contact the aHUS Registry Support Team
  - Email: [aHUS-Registry@SyneosHealth.com](mailto:aHUS-Registry@SyneosHealth.com)