

## **Systematic Identification and Mapping of Global Real-World Data Sources for Atypical Hemolytic Uremic Syndrome (aHUS)**

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### **Objective:**

To identify and describe global real-world data (RWD) sources for the rare disease of atypical hemolytic uremic syndrome (aHUS) to support future research.

### **Methods:**

A literature review was conducted to identify publications on RWD for aHUS using MEDLINE and EMBASE from the time of their inception until July 2020. Identified literature was critically reviewed and a list of unique aHUS data sources was derived. Detailed information for 43 variables was extracted including the type of data source, study design, population size, epidemiology, clinical, economic and humanistic burden, follow-up duration, efficacy outcomes and assessment of potential data access and linkage with other sources. A database abstraction tool in Excel format was developed to record information for each unique data source.

### **Results:**

A total of 2,302 publications were retrieved from the literature search and 148 unique data sources were identified; 16% were disease-specific and 84% covered a broader disease spectrum. More than one-third of the data sources covered Europe (35%), followed by Asia (29%), Americas (26%) and Oceania (5%), and 5% were multi-regional. More than half of these data sources (58%) had ≤50 patients and the most widely reported parameters were age (91%) and gender (76%) along with clinical parameters like platelet count (64%), serum creatinine (62%), hemoglobin levels (56%), dialysis (55%), all-cause mortality (55%), lactate dehydrogenase levels (LDH) (52%) and kidney transplant (41%). Some of the least reported parameters included hospital prescribed drugs (1%), health care resource utilization (1%) and quality of life (2%).

## Conclusions:

This project created a comprehensive list of published RWD sources for the rare disease aHUS that can potentially support future research. There are still critical gaps in the existing data highlighting the importance of future collaborations to collect granular and robust information to generate real-world evidence for better understanding of this rare disease.

**Word Limit:** 294/300 words

**Abstract submission deadline:** Tuesday 29 June 2021

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