RADeep Annual Baseline 2025

Key information

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What is the RADeep Annual Baseline ?

It is the yearly RADeep **survey** to estimate the ***accessible population*** of patients with Rare Anemia Disorders (RADs) in Europe.

Objectives:

- Assess the accessible RAD population in Europe.
- Evaluate a key EMA quality requirement for RWD: **representativeness**.
- Identify potential sources of bias in the registry.
- Measure registry coverage over time.
- Strengthen the scientific and regulatory value of RADeep.

What is the RADeep Annual Baseline ?

What is **representativeness**?

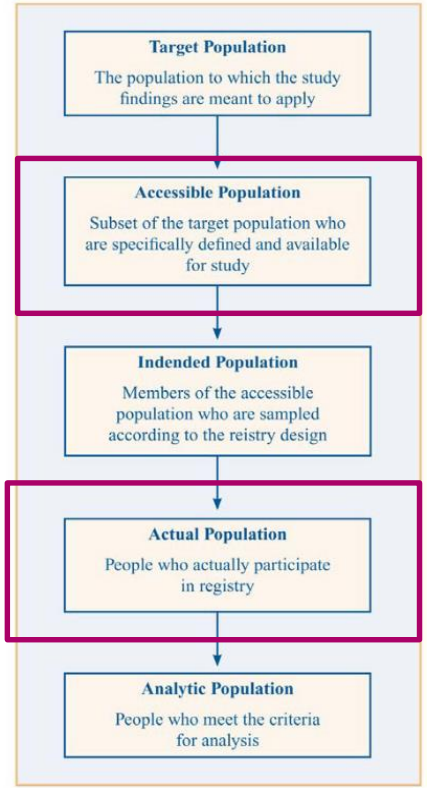
The extent to which the **RADeep registry** population reflects the real RAD patient population in Europe.

Why do we care about **representativeness**?

- Regulators evaluate it → **Key component of the EMA Data Quality Framework.**
- Ensures that registry data describes the general RAD population, not just subpopulations.
- Ensures **reliable** results and stronger RWE generation.

What is the RADeep Annual Baseline ?

Representativeness



Number of patients with RADs in regular follow-up at centres across EU-MS.

RADeep Annual Baseline

Number of patients with RADs enrolled in the registry.

RADeep

What is the RADeep Annual Baseline ?

Representativeness

RADeep → Actual population

RADeep Annual Baseline → Accesible population

What is collected?

- **Simple aggregated counts on** number of patients, age, sex, severity and treatment distribution.
- **NOT** patient individual level data.
- **Parameters** to be included in the survey agreed upon last **RADeep Data Access Committee** meeting on **18/09/2025:**
  - **Mandatory**
    - Total number of patients
  - **Optionals** → ***According to each center possibilities for data collection***
    - Number of adult/pediatric patients.
    - Number of male/female patients.
    - Number of patients per SCD genotype.
    - Number of patients under Hydroxiurea treatment.
    - Number of patients under regular transfusions.
    - Number of splenectomized patients.
    - Number of SCD patients with >= 2 VOE's in the last year.

Stratification by age (adult/pediatric)

Group of diseases included

Each data entry should report ***only*** the disease groups relevant to patients regularly followed at their center

Alpha-thalassemia and related diseases (ORPHA:275745)
Beta-thalassemia and related diseases (ORPHA:275749)
Unstable hemoglobin disease (hyper unstable) - Dominant beta thalassemia (ORPHA:231226)
Hemoglobinopathy (Other than thalassaemia, sickle cell disease, metahemoglobinemia and unstable hemoglobin disease) (ORPHA:68364)
Hereditary methemoglobinemia(ORPHA:621)
Unstable hemoglobin disease (moderate) (ORPHA:99139)
Hereditary elliptocytosis (ORPHA:288)
Hereditary spherocytosis (ORPHA:822)
Dehydrated hereditary stomatocytosis (ORPHA:3202)
Hereditary stomatocytosis (Other than dehydrated hereditary stomatocytosis) (ORPHA:98365)
Sitosterolemia (ORPHA:2882)
Rare constitutional hemolytic anemia due to a red cell membrane anomaly (Other than Hereditary Spherocytosis, Hereditary elliptocytosis, Hereditary Stomatocytosis)(ORPHA:98364)
Class A (Class 1 - chronic)) I glucose-6-phosphate dehydrogenase deficiency (G6PD) (ORPHA:466026)
Class B (acute-triggered) I glucose-6-phosphate dehydrogenase deficiency (G6PD) (ORPHA:466026)
Rare constitutional hemolytic anemia due to an enzyme disorder (Other than PKD, GPI, G6PD) (ORPHA:98369)
Hemolytic anemia due to glucophosphate isomerase deficiency (GPI)(ORPHA:712)
Rare constitutional hemolytic anemia due to pyruvate kinase deficiency (PKD) (ORPHA:766)
Sickle cell disease and related diseases (ORPHA:275752)
Congenital dyserythropoietic anemia (Other than type II) (ORPHA:85)
Congenital dyserythropoietic anemia type II (ORPHA:98873)
Thiamine-responsive megaloblastic anemia syndrome (ORPHA:49827)
Aceruloplasminemia (ORPHA:48818)
Congenital atransferrinemia (ORPHA:1195)
Constitutional sideroblastic anemia (Other than Severe congenital hypochromic anemia with ringed sideroblastic) (ORPHA:98362)
IRIDA syndrome (ORPHA:209981)
Microcytic anemia with liver iron overload(ORPHA:83642)
Severe congenital hypochromic anemia with ringed sideroblasts (ORPHA:300298)

Survey launching and key dates

- Opening on **2nd of March** until the **28th of April.**
- **REDCap credentials are not required**. Each appointed data entry will receive a **participation link** by email.
- Very simple and intuitive (Google Forms-like format).
- Why hosted in **REDCap**?
  - Provides secure GDPR-compliant data handling.
  - Supports continuous data quality monitoring.
  - Ensures complete traceability of data submissions.
- RADeep team available for support: **maximo.tartaglia@vhir.org.**

What will you see?

Survey Queue — Get link to my survey queue

In this queue you will find all the survey forms for each Disease Group in the RADeep Annual Baseline.

To open a form, click the **"Begin Survey"** button next to the Disease Group you treat at your center.

After you complete **all** forms for the disease groups managed at your institution, finish your participation by completing the **Survey Completion Confirmation** form at the bottom of this queue.

Status	Survey Title
Begin survey	**Sickle cell disease and related diseases**
Begin survey	**Alpha-thalassemia and related diseases**
Begin survey	**Beta-thalassemia and related diseases**
Begin survey	**Unstable hemoglobin disease (hyper unstable) - Dominant beta thalassemia**
Begin survey	**Hereditary methemoglobinemia**
Begin survey	**Unstable hemoglobin disease (moderate)**
Begin survey	**Hemoglobinopathy (Other than thalassaemia, sickle cell disease, metahemoglobinemia and unstable hemoglobin disease)**
Begin survey	**Hereditary elliptocytosis**
Begin survey	**Hereditary spherocytosis**
Begin survey	**Dehydrated hereditary stomatocytosis**
Begin survey	**Hereditary stomatocytosis (Other than dehydrated hereditary stomatocytosis)**
Begin survey	**Sitosterolemia**
Begin survey	**Rare constitutional hemolytic anemia due to a red cell membrane anomaly (Other than Hereditary Spherocytosis, Hereditary elliptocytosis, Hereditary Stomatocytosis)**
Begin survey	**Class A (Class 1 - chronic) I glucose-6-phosphate dehydrogenase deficiency**
Begin survey	**Class B (acute-triggered) I glucose-6-phosphate dehydrogenase deficiency**
Begin survey	**Rare constitutional hemolytic anemia due to an enzyme disorder (Other than PKD, GPI, G6PD)**
Begin survey	**Hemolytic anemia due to glucophosphate isomerase deficiency**
Begin survey	**Rare constitutional hemolytic anemia due to pyruvate kinase deficiency (PKD)**
Begin survey	**Congenital dyserythropoietic anemia (Other than type II)**

Do you have **Sickle cell disease and related diseases** patients in current regular follow up in your center?

(●) Yes ( ) No

* must provide value

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Please note:

The *total number of patients* is a mandatory field and must be reported if your center treats patients with this disease.

For each field, enter 0 if there are no cases to report or leave the field empty if the information is unknown (e.g. gender or treatment information is not available).

Total number of patients

Total Number of Patients	

Age

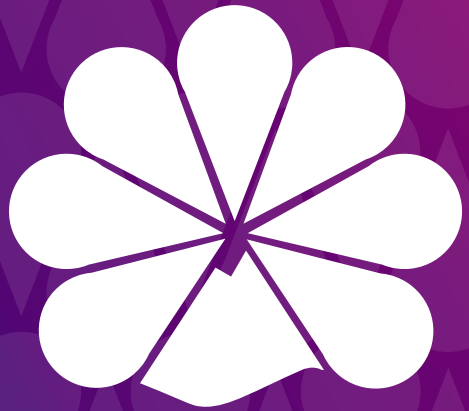
	Pediatric (< 18)	**Adult (≥18)**
Total Number of Patients		

Sex at birth

Sex at birth	**Pediatric (< 18)**	**Adult (≥18)**
Number of **male** patients		
Number of **female** patients		

Genotype

Number of patients with each genotype	**Pediatric (< 18)**	**Adult (≥18)**



RADeep

Thanks!

www.radeepnetwork.eu