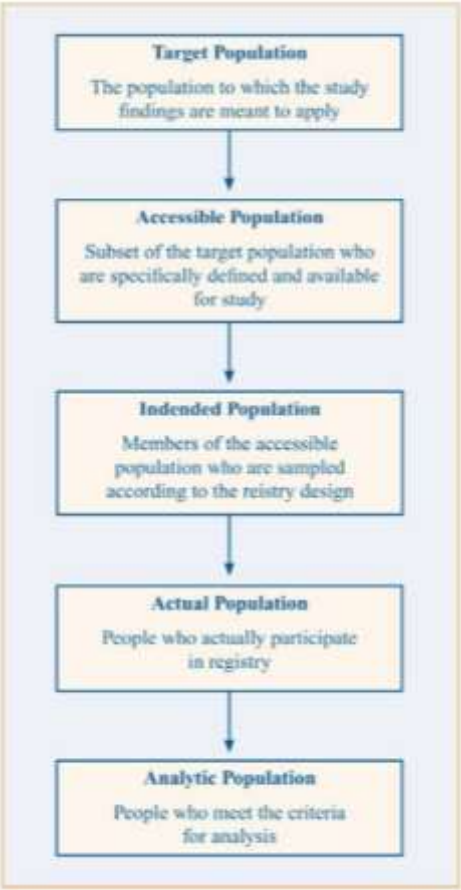
2025 RADeep Annual Baseline

What is the RADeep **Annual Baseline** ?



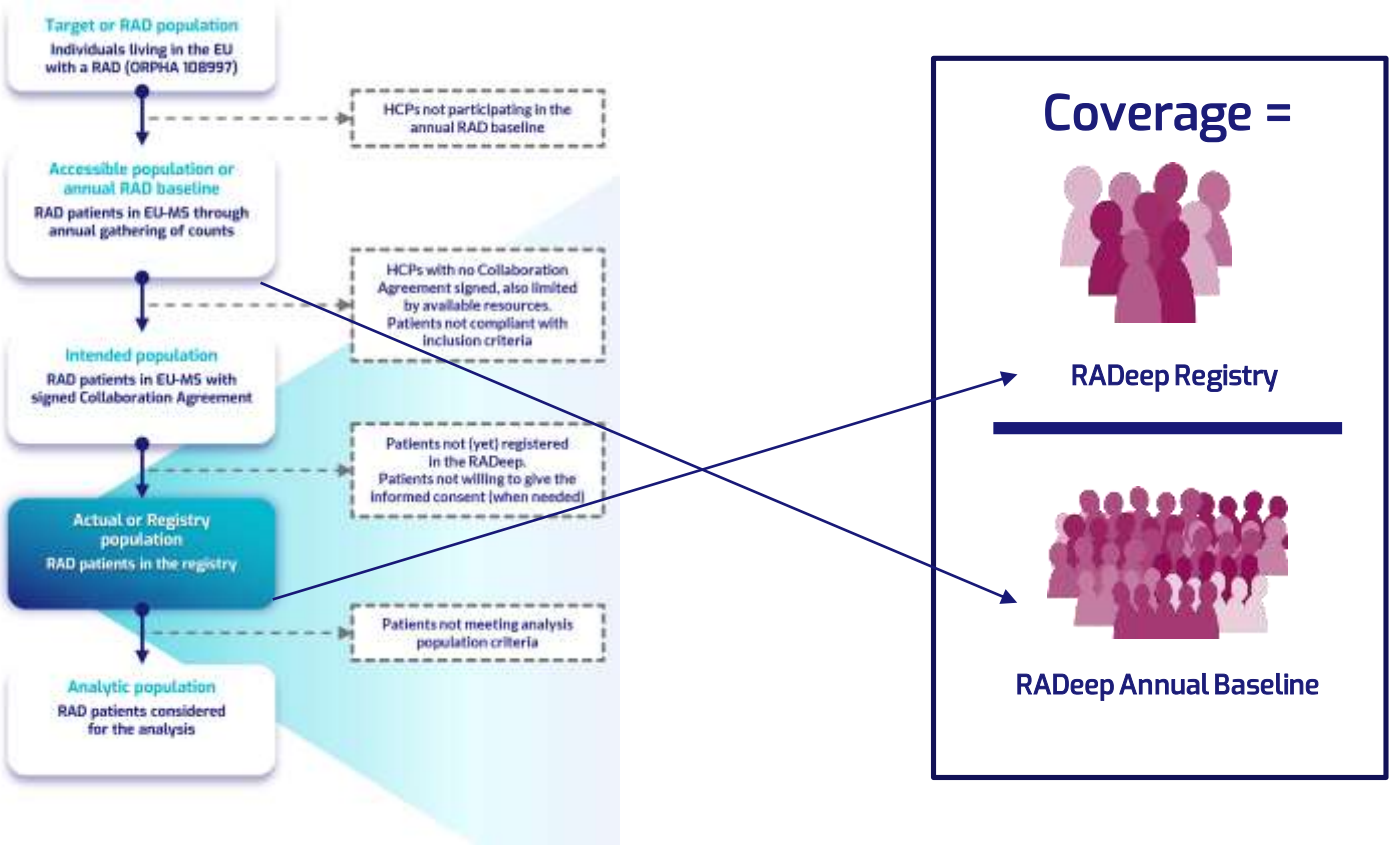
It is the yearly RADeep **survey** to estimate the ***accessible population*** , understood as *Number of patients with Rare Anemia Disorders (RADs) in regular follow-up at centres across EU-MS*.

Objectives:

- Estimate the accessible RAD population in Europe.
- Evaluate a key EMA quality requirement for Real World Data (RWD): **Representativeness.** Representativeness is defined as the data having the same characteristics as the whole it is meant to represent. "The extent to which the RADeep registry population reflects the real RAD patient population in Europe".
  - **Coverage**: % of patients registered / accessible population or Annual baseline
  - **Bias**: Risk to be registered based on individual characteristics (age, severity)
- Monitor registry coverage over time.
- Strengthen the potential of RADeep to generate Real World Evidence (RWE) for regulatory porpoises.

What is the RADeep **Annual Baseline** ?

Representativeness



What is collected?

- **Aggregated counts on** number of patients, age, sex, severity and treatment distribution.
  - NOT patient individual data
  - Aggregated counts are anonymyzed data → out of the scope of GDPR

- **Parameters** to be included in the survey agreed upon last **RADeep Data Access Committee** meeting on **18/09/2025:**
  - Total number of patients.
  - Number of adult/pediatric patients.
  - Number of male/female patients.
  - Number of patients per genotype (SCD).
  - Number of patients under Hydroxyurea treatment.
  - Number of patients under regular transfusions (SCD).
  - Number of patients under regular transfusions (yes/no) vs Number of splenectomized patients (yes/no).
  - Number of patients with >= 2 VOE's in the last year (SCD).

Stratification by age (adult/pediatric)

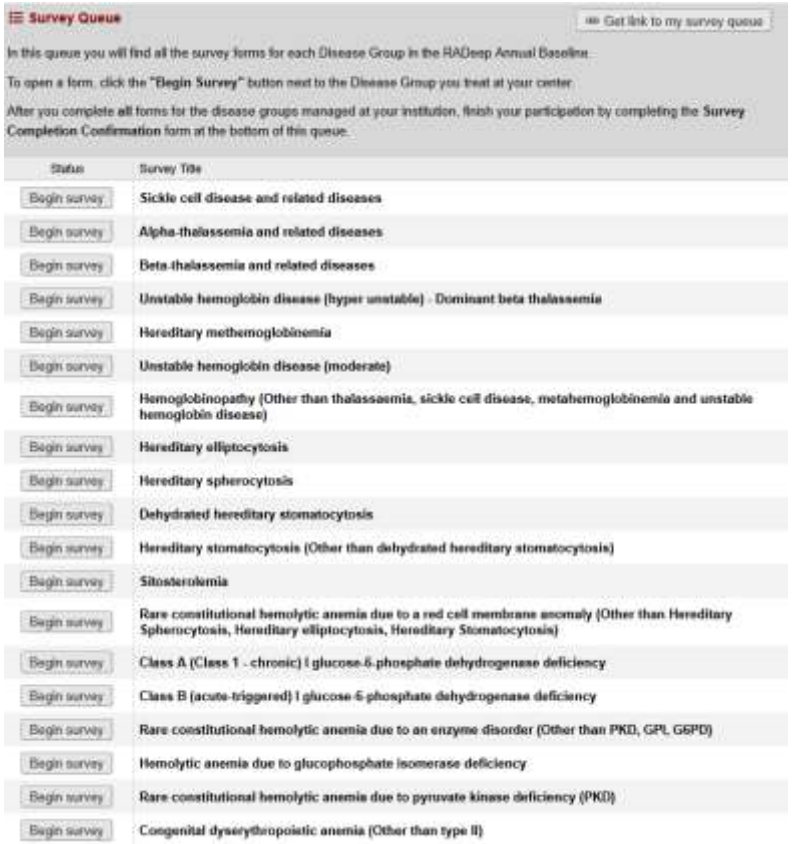
Group of diseases included

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 **March 9th**.
- **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 for DE information.
  - Supports continuous data quality monitoring.
  - Ensures complete traceability of data submissions.
- RADeep team available for support: alba.albert@vhir.org; sara.reidel@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

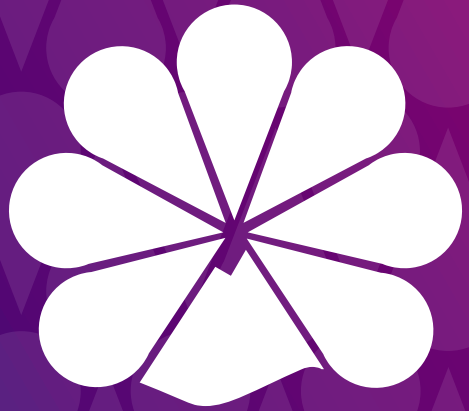
	Pediatric (< 18)	Adult (≥18)
Total Number of Patients		

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!

Any questions?

www.radeepnetwork.eu