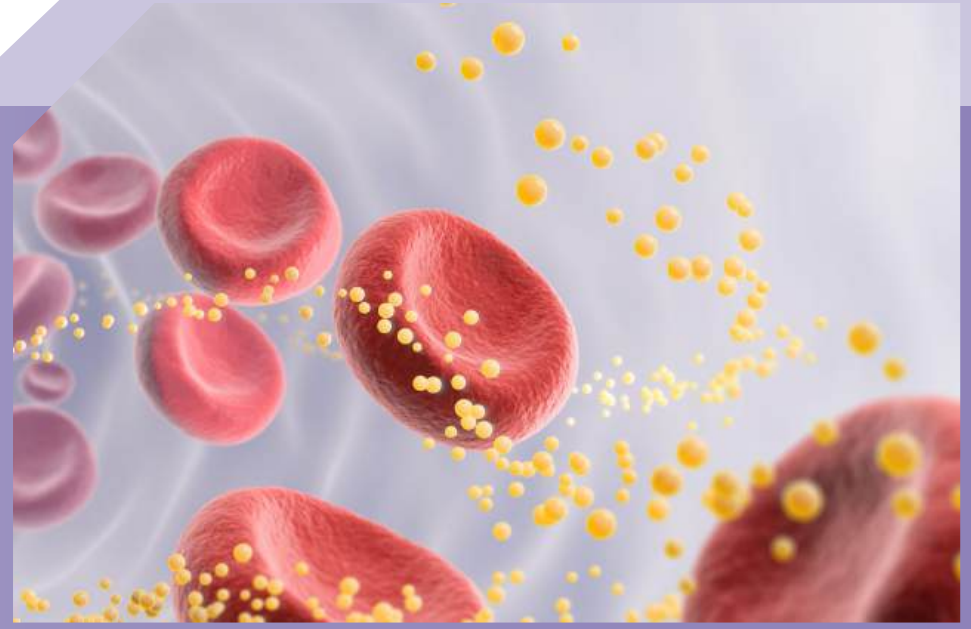
Protheragen
Rare Blood Disorders

Customized Solutions for Rare Blood Disorder Diagnostic & Therapeutic Development



Contact Us

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www.protheragen.us/blood-disorders/

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Overview

About Us

As a leading research services provider, Protheragen specializes in providing customized diagnostic and therapeutic development solutions for rare blood disorders to meet the specific needs of researchers. Our dedicated team is committed to driving innovation and advancements to improve the detection, understanding and treatment of these complex diseases.

- Diagnostic Development
- Disease Model Development
- Therapeutic Development
- Preclinical Research

OUR ADVANTAGES

Precision and Innovation: Your Trusted Diagnostic & Therapeutic Research Partner

- Specialized Expertise
- Cutting-edge Technology
- Innovative Therapies
- Comprehensive Services

LEADING RESEARCH SERVICES PROVIDER

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Disease Areas of Focus

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Cutting-edge Research on Multiple Rare Blood Disorders

Rare blood disorders represent a diverse set of complex conditions affecting the blood and its components. These disorders often involve abnormalities in the production, function, or structure of blood cells, leading to a range of symptoms such as anemia, clotting problems, and compromised immunity. With a focus on precision and innovation, Protheragen is committed to providing cutting-edge diagnostic and therapeutic development solutions for a variety of rare blood diseases.

Myeloproliferative Disorders (MPDs)

Bone Marrow Failure Syndromes (BMFS)

Lymphoproliferative Disorders (LPDs)

Bleeding Disorders

Anemia

Here are some examples of rare blood disorders for our focus:

- Chronic Neutrophilic Leukemia (CNL)
- Essential Thrombocythemia (ET)
- Myelofibrosis (MF)
- Polycythemia Vera (PV)
- Paroxysmal Nocturnal Hemoglobinuria (PNH)
- Telomere Biology Disorders (TBDs)
- Adult T-Cell Leukemia/Lymphoma (ATLL)
- Diffuse Large B-Cell Lymphoma (DLBCL)
- Non-Hodgkin Lymphoma (NHL)
- Acquired Hemophilia A (AHA)
- Alpha-Thalassemia (A-THAL)
- Others

One-stop Solutions

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Protheragen specializes in preclinical research for rare blood disorders, offering a comprehensive suite of services encompassing diagnostics, treatments, disease model development, and preclinical drug evaluations. Particularly in the realm of therapeutic development, we provide end-to-end solutions spanning target identification, candidate drug discovery, and drug development.

Diagnostic Development

- Biomarker Development
- *In Vitro* Diagnostic (IVD) Kit Development
- Point-of-Care Testing (POCT) Development
- Companion Diagnostic Development

Therapeutic Development

- Small Molecule Drug
- Cell Therapy
- Gene Therapy
- Therapeutic Antibody
- Therapeutic Peptide
- Therapeutic Protein

Disease Model Development

- Cell-Based Model Development Service
- Organoid Model Development Service
- Animal Model Development Services

Preclinical Research

- Pharmacodynamic (PD) Research Services
- Pharmacokinetic (PK) Research Services
- Toxicology Research Services

Clinical Trial Services

- Investigator Initiated Trial (IIT) Services
- IND/NDA/BLA Application
- Patient Recruitment
- Biostatistics Services
- Other Services

Diagnostic Development

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Diagnosing rare blood disorders poses significant challenges, necessitating the utilization of specialized tools for precise disease identification. Protheragen stands at the forefront of tackling the intricate diagnostic obstacles associated with rare blood disorders, offering clients extensive diagnostic development solutions to streamline the effective management of these conditions.

Biomarker Development Service

Dedicated to identifying and validating key molecular markers required for early detection and accurate diagnosis of rare blood diseases, Protheragen provides high-quality biomarker development services with cutting-edge technology and rigorous validation processes.

POCT Development Service

Protheragen offers portable testing solutions for rare blood diseases, revolutionizing the accessibility of diagnostics. We assist in developing tools with real-time diagnostic capabilities to facilitate rapid decision making in resource-limited settings.

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IVD Kit Development Service

In vitro diagnostic (IVD) kit development services are designed to create highly sensitive and specific diagnostic kits for rare blood diseases. From concept to deployment, we always follow strict quality standards and regulatory requirements to ensure the accuracy and reliability of products.

Companion Diagnostic Development

By correlating specific biomarkers with therapeutic response profiles, we tailor companion diagnostic solutions to optimize therapies, monitor therapeutic outcomes, and minimize adverse reactions to the greatest extent possible. These well-validated tools advance personalized medicine.

Therapeutic Development

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Service Workflow

At Protheragen, we provide comprehensive end-to-end therapeutic development solutions for rare blood disorders, guiding your drug from discovery to commercialization. Our integrated expertise in biomarker-driven target validation, precision drug design, and preclinical evaluation ensures seamless translation of research findings into approved therapies.

Target Identification → Drug Discovery → *In Vitro* Studies → *In Vivo* Evaluation

Targets	Biological Role	Associated Rare Blood Disorders
JAK2	Tyrosine kinase signaling	Polycythemia Vera (PV), Myelofibrosis
BCR-ABL1	Oncogenic fusion kinase	Chronic Myeloid Leukemia (CML)
Complement C5	Terminal complement activation	Paroxysmal Nocturnal Hemoglobinuria (PNH)
TMPRSS6	Hepcidin regulation	Iron Deficiency Anemia
EPO Receptor	Erythropoiesis regulation	Congenital Erythropoietic Porphyria
CD19/CD20	B-cell surface markers	Rare B-cell malignancies
VWF/ADAMTS13	Von Willebrand Factor cleavage	Thrombotic Thrombocytopenic Purpura (TTP)
BCL-2	Anti-apoptotic protein	Waldenström Macroglobulinemia

Disease Model Development

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Precision disease models are indispensable tools for rare blood disorder research, enabling reliable drug efficacy and safety evaluation, and translation of discoveries into clinically relevant therapies. Protheragen is dedicated to building accurate disease models that realistically reproduce the complex pathophysiology of rare blood disorders to accelerate your therapeutic development.

Cell-based Model Development

- Cell Line Development
- Primary Cell Development
- iPSC Development

Organoid Model Development

- ASC-derived Organoid
- ESC-derived Organoid
- iPSC-derived Organoid
- Patient-derived Organoid

Animal Model Development

- Genetically Engineering Model
- Humanized Animal Model
- Induced Disease Model
- Surgery Model
- Syngeneic Model
- Xenograft Model

Disease Model Development

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Customized Animal Model Development

- Protheragen specializes in developing highly predictive animal models for rare blood disorders, enabling robust evaluation of your therapeutics in pharmacokinetics, pharmacodynamics, and toxicology studies.
- These meticulously designed models offer a controlled experimental environment to help bridge the gap between research findings and clinical success.

LEADER IN ANIMAL MODEL DEVELOPMENT

Model Names	Rare Blood Disorders	Model Types
Flvcr1 CKO Model	Diamond-Blackfan Anemia (DBA)	Genetically Engineering Model
F8 KO Model	Hemophilia A	Genetically Engineering Model
F9 KO Model	Hemophilia B	Genetically Engineering Model
Itga2b Mu Model	Glanzmann Thrombasthenia	Genetically Engineering Model
Fanca KO Model	Fanconi Anemia (FA)	Genetically Engineering Model
L540/L540Cy PDX Model	Hodgkin Lymphoma (HL)	Xenograft Model
A20 PDX Model	Non-Hodgkin Lymphoma (NHL)	Xenograft Model
Jeko-1 PDX Model	Mantle Cell Lymphoma (MCL)	Xenograft Model
3-methylcholantrene (3-MC) Induced Model	Lymphoblastic Lymphoma (LBL)	Induced Model
Mesenteric Vessel Injury Model	Von Willebrand Disease (VWD)	Surgery Model

For more information about our animal model development services for rare blood disorders, please contact us.

Case Study-Humanized Mouse GVL Model Development

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Challenges in AML Treatment: Acute myeloid leukemia (AML) is the most common acute leukemia in adults, and its treatment relies on allogeneic stem cell transplantation (alloSCT) and its graft-versus-leukemia (GVL) effect. However, GVL failure and AML relapse caused by leukemic immune escape remain the main causes of mortality after transplantation.

GVL Model: The GVL model is a humanized mouse system that recapitulates the clinical dynamics of allo-HSCT by co-engrafting patient-derived leukemia xenografts with immune cells from healthy donors (*e.g.*, G-CSF-mobilized PBMCs or HSCs) into NSG mice.

Model Application: The GVL model is designed to reflect the intricate interactions between human leukemia and the immune system, providing a critical preclinical platform for studying leukemia immune escape mechanisms and optimizing alloSCT therapy. By assessing both GVL effect and graft-versus-host disease (GVHD), the model can discover new targets, test immunotherapies, and develop personalized strategies to prevent relapse after transplantation.

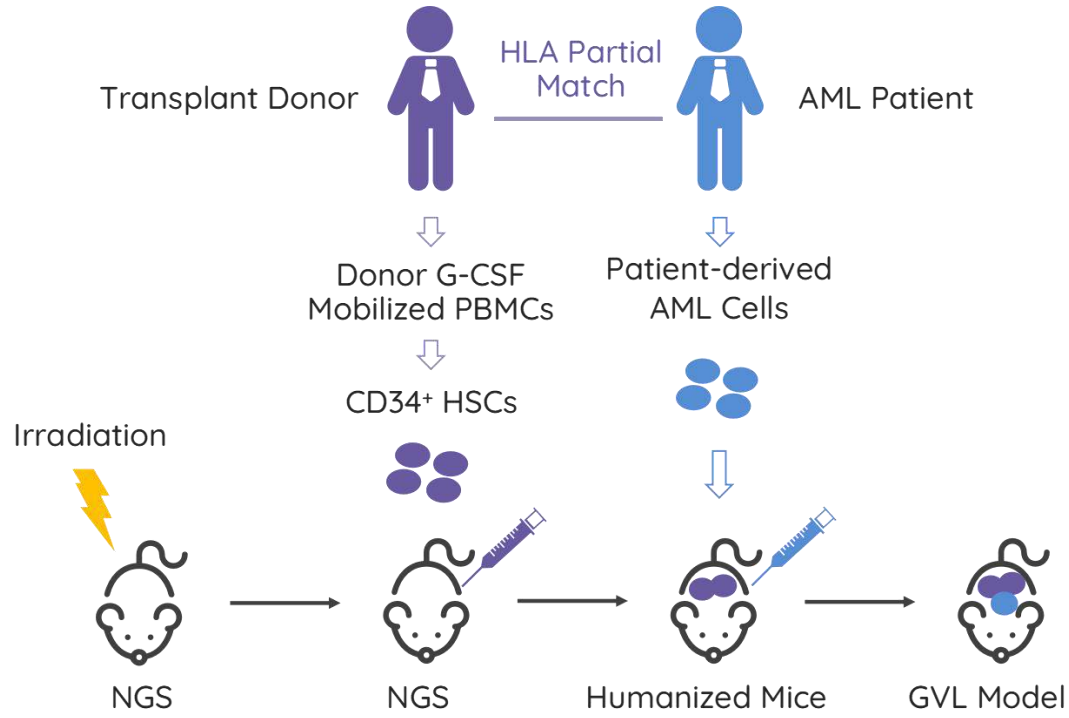

Fig.1 Development process of the humanized mouse GVL model.

12 weeks after the NSG mice received the donor HSC injection, our scientists injected the patient's AML cells into the mouse femur. Four weeks after the transplant, leukemia cells expressing CD33 were detected in the blood. At 12 weeks, their content increased to 70% and infiltrated other organs (bone marrow, spleen, liver, lungs). These results are consistent with clinical observations, indicating the successful engraftment of patient-derived AML in hu-NSG mice.

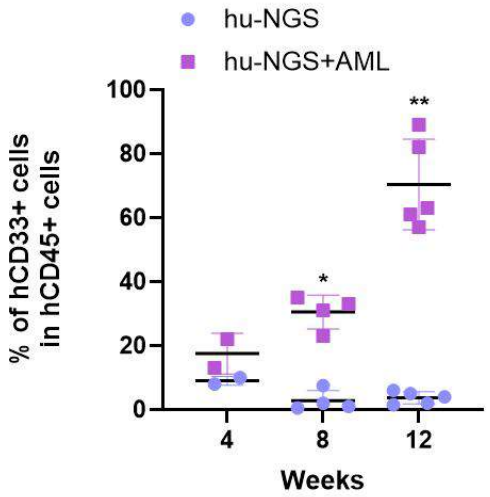

Fig.2 The proportion of CD33-positive lymphoma cells in the peripheral blood of hu-NSG mice was detected at 4, 8, and 12 weeks after AML transplantation.

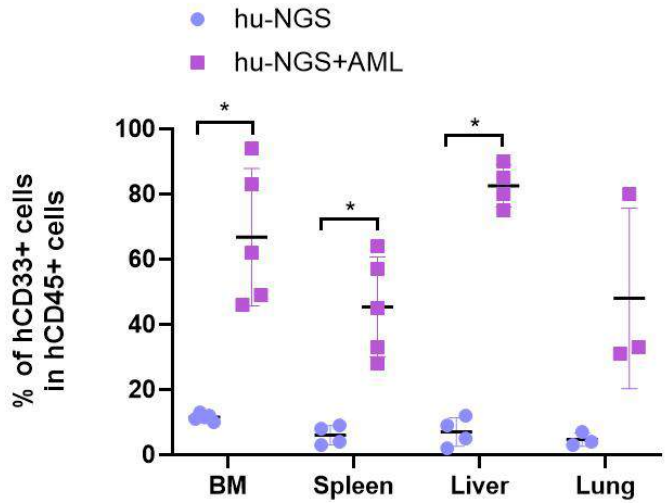

Fig.3 The distribution of CD33-positive lymphoma cells in different organs was detected at 12 weeks after AML transplantation.

Case Study-Leukemic Immune Escape in Hu-GVL Mice

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Experimental Purpose

In clinical cases, upregulation of some immunosuppressive pathway molecules is often found in patients with AML relapse after alloSCT. To verify whether the hu-GVL model can reproduce the clinical characteristics of these patients, our scientists compared the expression of immunosuppressive pathway molecules on CD8-positive T cells in the peripheral blood of hu-GVL and hu-NSG (no AML cells transplanted) mice.

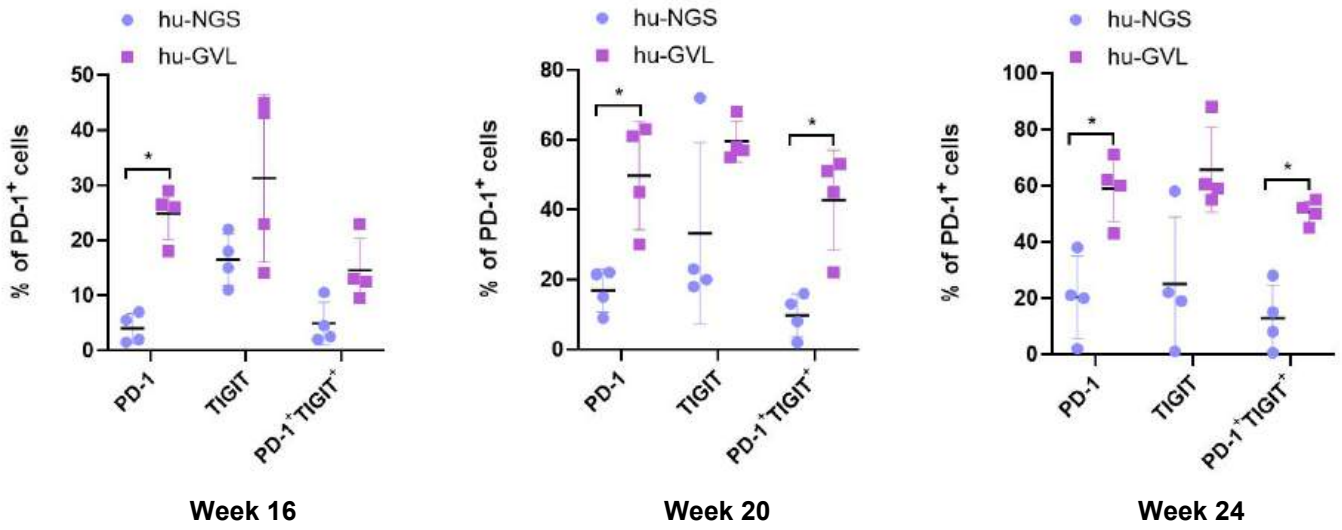

Fig.4 The proportion of PD-1 positive, TIGHT positive, and PD-1/TIGHT double positive cells on the surface of CD8 T cells in the peripheral blood of hu-NSG and hu-GVL mice.

Experimental Results

Following transplantation of AML cells, hu-GVL mice exhibit a higher proportion of cells expressing PD-1 alone and PD-1/TIGIT dual positivity at most time points. Furthermore, we assessed bone marrow cells from hu-GVL mice and NSG mice transplanted with AML cells, revealing a significant increase in the expression levels of PD-L1 and CD155 (TIGHT ligand) in the hu-GVL model, while the expression of HLA-DR is markedly reduced. These findings align consistently with observations in clinical patients.

Preclinical Research

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Pharmacodynamic (PD) Research Services

- Dose-Response Studies
- Biomarker Identification
- Mechanism of Action Studies
- Efficacy Testing
- Drug Screening
- Drug Combination Studies

Pharmacokinetic (PK) Research Services

- Metabolism Studies
- Permeability Studies
- Drug Excretion Studies
- Tissue Distribution Studies
- Bioavailability and Bioequivalence Studies
- Metabolite Profiling and Identification Studies

Toxicology Research Services

- General Toxicology Assessments
- Genetic Toxicology Evaluations
- Specialized Toxicology Assessments
- Toxicokinetic Analyses

Clinical Research Services

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CLINICAL RESEARCH

Protheragen offers a full suite of customized services for clinical trials in the rare blood disorder field. Our unique value lies in our commitment to ensuring the success of blood disorder-focused clinical trial programs through a steadfast emphasis on quality, strict adherence to regulations, and a customer-centric approach.

- Investigator Initiated Trial (IIT) Services
- Medical Writing Services
- IND/NDA/BLA Application Strategy
- Patient Recruitment
- Biostatistics and Programming
- Quality Management Services
- Medical Monitoring Services
- Safety and Pharmacovigilance Services
- Data Management Services
- Project Management Services

Deep Therapeutic Expertise

Technology-Driven Efficiency

End-to-End Solutions

Regulatory & Compliance Mastery

Featured Products

Protheragen
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Protheragen delivers premium, compliance-ready biological products for rare blood disease research, including rigorously validated biological samples, clinical-grade cell products, and GMP-compliant coagulation factor related products. Our products meet stringent quality standards and are traceably sourced to ensure reliability for both diagnostic development and therapeutic research.

Biological Samples

Tailored to your specific requirements, we ensure you receive precise samples essential for researching rare blood diseases. Our sampling process adheres to the highest standards and ethical guidelines to guarantee the integrity and reliability of the samples.

- Blood Samples
- Bone Marrow Aspirates
- Tissue Samples

Cell Products

Protheragen is committed to providing a broad range of cell products to meet the diverse needs of researchers worldwide.

- Hematopoietic Stem Cells
- Red Blood Cells
- White Blood Cells
- Platelets
- Mononuclear Cells (MNCs)
- Plasma Cells
- Dendritic Cells

Coagulation Factor Related Products

Committed to innovation and top-notch quality, our company ensures that every coagulation factor related product is carefully designed to maintain excellent efficacy, safety and compliance.

- Recombinant Coagulation Factors
- Factor Concentrates
- Coagulation Factor Inhibitors
- Coagulation Factor Antibodies
- Plasma-Derived Factor

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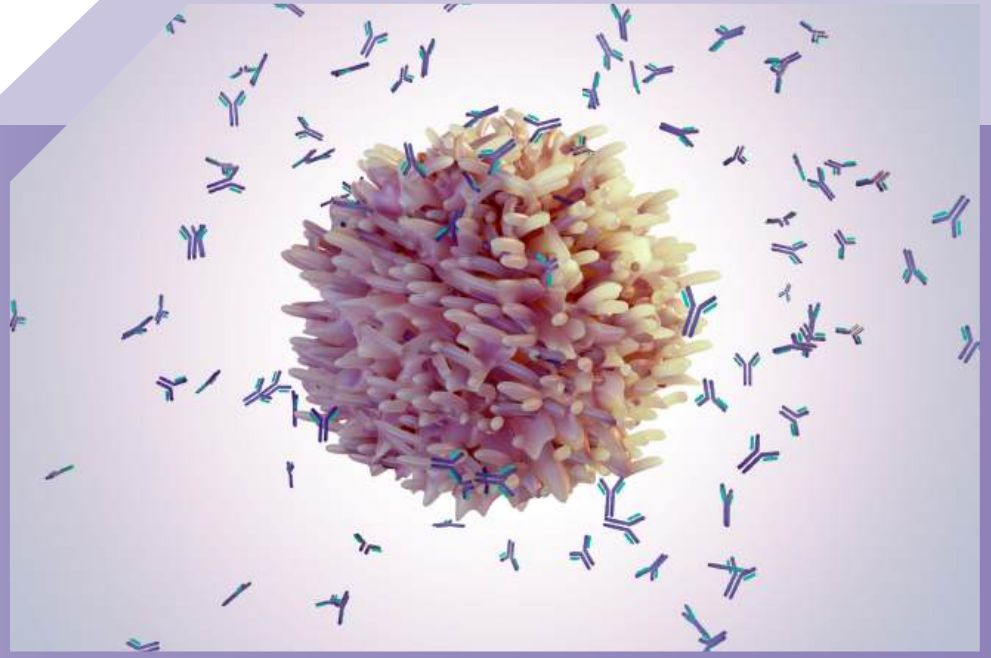
Your Trusted Partner in Rare Blood Disorder Diagnostic & Therapeutic Development



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