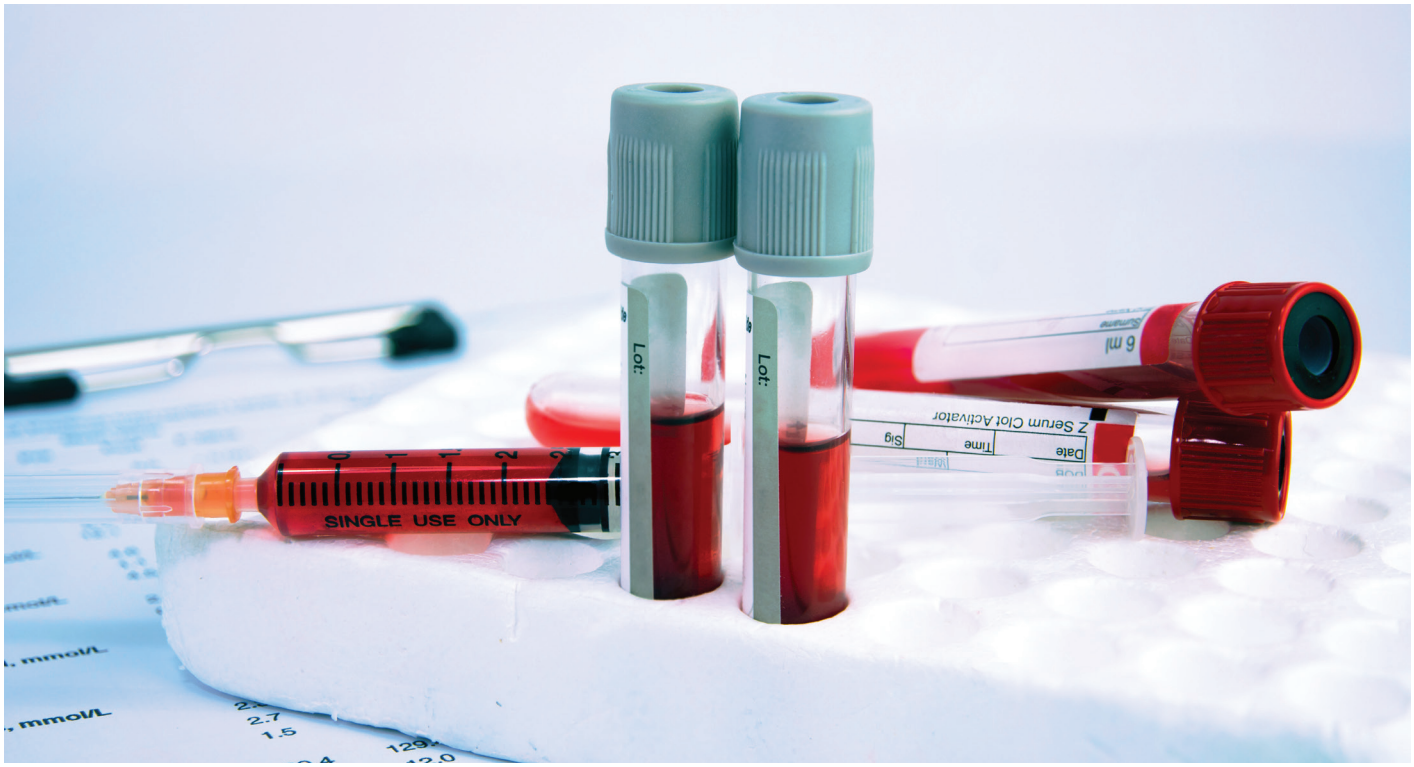
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Developing Medicines To Address Unmet Needs In Hematological Diseases



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Developing Medicines To Address Unmet Needs In Hematological Diseases

By L. Andres Sirulnik, MD, PhD

Over the last few decades, progress in experimental molecular biology has transformed medicine by shedding light on disease pathogenesis and fostering technological breakthroughs. The field of hematology – often the proving ground of bench-to-bedside discovery – is at the forefront of this progress. Although the complexities of blood diseases have made them historically difficult to treat, the industry continues to make great strides; in the last two decades, for example, median survival has doubled for follicular lymphoma, a type of blood cancer that affects white blood cells. Encompassing diseases of the blood, bone marrow, immune, hemostatic, and vascular systems, the field of hematology hosts numerous unmet medical needs that have the potential to be addressed by novel therapies in development. Building on its research, Regeneron is investing in its recently formed clinical hematology group to translate scientific advances into medicines that can potentially change patients' lives.

For over 30 years, Regeneron's scientists have been at the forefront of discovery in molecular biology and preclinical hematology: seminal discoveries include identifying and cloning the ciliary neurotrophic factor (CNTF) receptor and demonstrating its similarity to interleukin(IL)-6, characterizing cell-type dependent interactions of CNTF with JAK-TYK kinases, discovering angiopoietin-1 and its partnership with the TIE2 receptor to promote angiogenesis, and developing vascular endothelial growth factor (VEGF) binding peptides. By 2020, using the knowledge gained from basic biological research, Regeneron had advanced multiple

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investigational drug candidates to clinical development. With the clinical-phase pipeline growing, Regeneron united classical hematology and hematology-oncology into one team and re-imagined how a hematology clinical development group could accelerate programs and with the goal to bring medicines to patients faster.

Classical hematology addresses disorders such as anemias, clotting disorders, and other nonmalignant blood diseases, while **hematology-oncology** addresses the myriad of blood cancers such as multiple myeloma and lymphoma. The two disease areas share a great deal of biology and are often treated by the same clinicians – hematologists and oncologists frequently handle cases interchangeably and physicians specializing in hematology and oncology are often double-board certified. Yet, the two sets of diseases are sometimes split in the drug development industry, with separate groups handling classical hematology and hematology-oncology. Regeneron realized the strengths of having one team managing the clinical development of the full hematological pipeline; when Regeneron formed its hematology group, the company united the teams studying both sets of diseases. The clinical hematology team now mirrors how the wide array of hematological diseases are treated.



Regeneron made its name with leading-edge scientific research that brings deep knowledge of disease biology and unique animal models.

Building A Diverse Development Team With Broad Capabilities

Regeneron's hematology program embodies the philosophy of its broader development organization. The success of **Regeneron is built on end-to-end capabilities** to discover, develop, and manufacture medicines. These diverse functions of Regeneron further set the organization apart. From target discovery and preclinical research to toxicology and first in human (FIH) studies, Regeneron has the end-to-end capabilities that rival the speed, rigor, and innovation of the most seasoned players in the industry. Regeneron made its name with leading-edge scientific research that brings deep knowledge of disease biology and unique animal models.

That's how, in recent years, Regeneron has been able to build a broad hematology pipeline: by following the science and not centering around building franchises. Regeneron focuses on the biology first and promotes flexibility in applying the science across diseases and disciplines. This approach, coupled with a deep understanding of hematological diseases, is how Regeneron stands out.

Attracting and retaining the industry's top talent has been central to the success of the group. Recognizing that its people are the engine behind successfully delivering medicines to patients, Regeneron is investing in its development team with a focus on hiring physicians and scientists from a diverse range of backgrounds – reflecting the strongly held belief that **diversity of opinion, thought, experience and expertise fuel the success of Regeneron**. This diversity among teams is what powers Regeneron at every step of the drug development journey, allowing each group to work in harmony with other teams.

It's this special combination of diverse people,

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functions and teams that allows Regeneron to do incredible work, like that spotlighted during COVID-19.

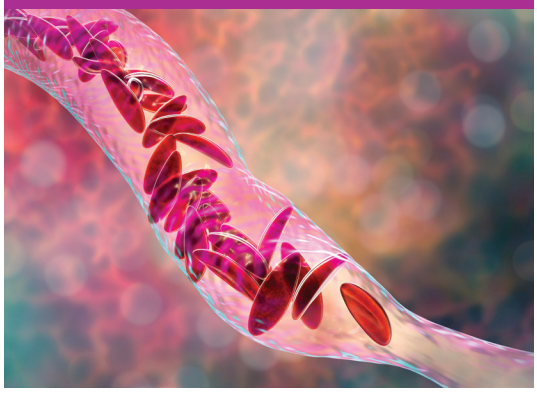
Aspiring To Transform Treatment With Novel Approaches

Using a science-led approach has established Regeneron as a leader in researching and developing treatments for a variety of medical conditions including eye diseases, allergic and inflammatory diseases, cancer, cardiovascular and metabolic diseases, pain, infectious diseases, and rare diseases. Regeneron is positioned to potentially address major unmet medical needs across hematology, especially in classic hematology, which is historically under-funded. The hematology development group is working to translate science into potential breakthrough medicines to address unmet medical needs for patients.

These investments have yielded candidates for classical hematological diseases. In aplastic anemia – a serious condition defined by the inability of the bone marrow and stem cells to produce enough blood cells – Regeneron is researching the potential of using a monoclonal antibody to IL2Rγ. This therapeutic approach modulates pathogenic T cells and may potentially offer a new tool to treat T cell-mediated diseases, including Graft-versus-Host Disease and various autoimmune diseases. Regeneron is also investigating targeting the complement system with its investigational anti-C5 monoclonal antibody, pozelimab, for conditions like paroxysmal nocturnal hemoglobinuria (PNH), where there remains a significant unmet need to reduce treatment burden for patients. Pozelimab blocks the complement pathway, which when dysregulated, leads to the abnormal destruction of red blood cells, leading to the symptoms of PNH and other complement-mediated diseases. A recently initiated combination trial of Pozelimab with Alnylam's RNA interference (RNAi) therapy for PNH, cemdisiran, will allow Regeneron to research and develop complementary mechanisms of action for a potentially enhanced effect.

In hematological malignancies, Regeneron is exploring the use of a bispecific platform to treat blood cancers. Bispecific antibodies are a new, innovative class of medicines. Regeneron has advanced multiple investigational CD3 bispecifics, which are designed to bridge T-cells and tumor cells and promote T-cell activation and killing of cancer cells with incredible precision. The hematology development team is further investigating clinical proof of concept for the platform with its investigational candidates odronextamab (targeting CD20) and REGN5458 (targeting BCMA), setting the stage for further work to treat malignancies.

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The T cell engager program is a testament to the power of Regeneron's antibody platforms, and the company is continuing to explore other ways of researching potential treatments for hematological diseases. Regeneron's commitment to fostering innovation in the field is illustrated by its partnership with Intellia Therapeutics, a company focused on CRISPR-based gene editing.

In 2021, Regeneron and Intellia released the first ever clinical

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data of *in vivo* CRISPR genome editing in humans. The proof of concept in hereditary amyloidosis was a landmark for the medical field and helped amplify Regeneron and Intellia's efforts to investigate potential treatments for hereditary amyloidosis and other hereditary conditions such as hemophilia A and B.

Investing In Teams To Help Improve Lives

The range of novel, impactful approaches to researching and developing the treatment of hematological diseases in development at Regeneron demonstrates the ingenuity of its research teams. The research teams work hand-in-hand with the development teams, paving the path for exciting science to evolve into potential approved medicines.

Regeneron established the clinical sciences hematology group to ensure it has the powerful, diverse development team needed to meet its goal of getting therapies to patients quickly without compromising on quality. It's a team in which Regeneron continues to invest because of its powerful capacity to move breakthrough drug candidates through clinical studies and potentially change the lives of millions of patients.

Investigational therapies featured, and their safety and efficacy, have not been fully evaluated by any regulatory authority. This information is what is known or hypothesized about the biologic mechanisms of these antibodies and is not intended to convey clinical efficacy or safety.

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Regeneron is a leading biotechnology company that invents life-transforming medicines for people with serious diseases. Founded and led for over 30 years by physician-scientists, our unique ability to repeatedly and consistently translate science into medicine has led to nine FDA-approved treatments and numerous product candidates in development, almost all of which were homegrown in our laboratories. Our medicines and pipeline are designed to help patients with eye diseases, allergic and inflammatory diseases, cancer, cardiovascular and metabolic diseases, pain, hematology, infectious diseases and rare diseases.

For additional information about the company, please visit www.regeneron.com or follow @Regeneron on Twitter.

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