WFH GLOBAL FORUM

14th WFH Global Forum
on Advances and Access to Safe Treatment Products for Bleeding Disorders

SUMMARY

November 13-14, 2025
Montreal, Canada

WFH
WORLD FEDERATION OF HEMOPHILIA
FÉDÉRATION MONDIALE DE L'HÉMOPHILIE
FEDERACIÓN MUNDIAL DE HEMOFILIA

Summary of the 14th WFH Global Forum on Advances in Treatments and Technologies for Bleeding Disorders

Please note: This material is intended for general information only. The World Federation of Hemophilia does not endorse particular treatment products or manufacturers; any reference to a product name is not an endorsement by the WFH. The WFH is not a regulatory agency and cannot make recommendations relating to safety of manufacturing of specific blood products. For recommendations of a particular product, the regulatory authority in a particular country must make these judgments based on domestic legislation, national health policies, and clinical best practices.

The WFH does not engage in the practice of medicine and under no circumstances recommends particular treatment for specific individuals. Any treatment must be designed according to the needs of the individual and the resources available.

Published by the World Federation of Hemophilia

World Federation of Hemophilia
1184 rue Sainte-Catherine Ouest, Suite 500
Montreal, Quebec H3B 1K1
CANADA
Tel.: (514) 875-7944
E-mail: wfh@wfh.org
Internet: www.wfh.org

Acknowledgements
The World Federation of Hemophilia gratefully acknowledges funding support for Global Forum 2025 from CSL, Pfizer, Sanofi, and Sobi; with additional support from: Biomarin. Only through such support and partnerships can the WFH continue to provide important global interchange on issues related to blood product safety, supply, and access.

AI Analysis

Safety and surveillance

Steven Grossman
President, HPS Group, LLC

Dr. Thomas Kreil
Associate Professor of Virology, Medical University of Vienna Vice President, Global Pathogen Safety, Takeda

Moderator: Mark Skinner
President and Chief Executive Officer, Institute for Policy Advancement Ltd, U.S.A.

Summary

The session on safety and surveillance highlighted the significant changes in the U.S. healthcare system and their implications for global pathogen safety. Mark Skinner introduced the session, noting the importance of understanding the current threats to patients, including supply and access to treatment and pathogen transmission. Thomas Kreil presented a detailed overview of the global pathogen safety landscape, emphasizing the continuous threat posed by viruses and the need for robust safety measures. He explained the historical context of virus exposure and the advancements in virus reduction capacity, highlighting the critical role of classical virology in ensuring safety. Kreil also discussed the potential risks associated with emerging infectious diseases and the importance of science-based balancing of safety and availability.

Steven Grossman provided an analysis of the current public health environment in the U.S., focusing on the challenges posed by the new administration's policies. He highlighted the breakdown in the sense of common good and the significant changes in key agencies such as the CDC, NIH, and FDA. Grossman discussed the impact of budget cuts and policy changes on research funding and regulatory processes, emphasizing the need for continuous vigilance and adaptation. He also mentioned the importance of aligning with the administration's priorities, such as unmet medical needs, to secure funding and support.

The speakers agreed on the critical role of surveillance and monitoring in ensuring safety, with Kreil emphasizing the importance of classical virology and Grossman highlighting the challenges posed by policy changes. They both stressed the need for a science-based approach to balancing safety and availability, given the continuous threat of emerging infectious diseases. Kreil's insights into the historical context and advancements in pathogen safety provided a comprehensive understanding of the current landscape, while Grossman's analysis of policy changes offered a practical perspective on navigating the evolving regulatory environment.

The session also addressed the implications of recent U.S. regulatory and budgetary changes on global safety and surveillance. Kreil noted that the CDC's capabilities are unmatched globally and that their pullback could have significant consequences. Grossman echoed this sentiment, highlighting the importance of the CDC's work and the need for advocacy to ensure the continuation of critical programs. They discussed the potential impact of changes in grant funding processes at the NIH and the importance of adapting research proposals to align with current priorities.

In conclusion, the session underscored the importance of continuous vigilance and adaptation in pathogen safety and surveillance. Kreil's detailed overview of virus exposure and safety measures, combined with Grossman's analysis of policy changes, provided a comprehensive understanding of the current challenges and opportunities. The speakers emphasized the need for science-based approaches to ensure safety and availability, given the evolving threats posed by emerging infectious diseases and regulatory changes.

The session highlighted the critical role of advocacy in securing support for pathogen safety and surveillance programs. The speakers stressed the importance of engaging with both the administration and Congress to ensure the continuation of vital programs. They also discussed the need for strategic adaptation of research proposals to align with current priorities, emphasizing the importance of unmet medical needs in securing funding and support. Overall, the session provided valuable insights into the challenges and opportunities in pathogen safety and surveillance, emphasizing the need for continuous vigilance, adaptation, and advocacy.

Takeaways

Continuous Threats from Viruses

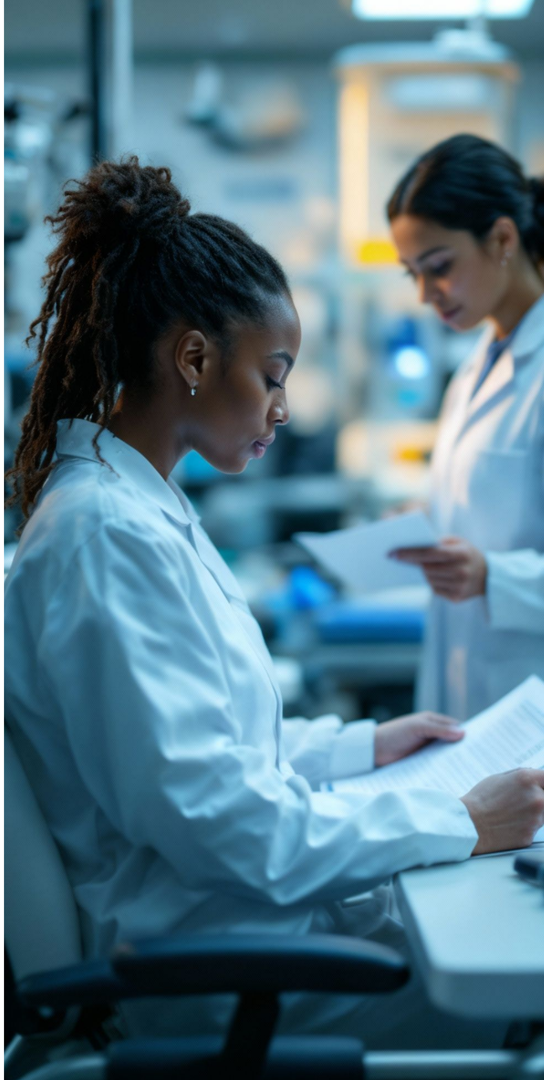
Thomas Kreil emphasized the ongoing threat posed by viruses and the need for robust safety measures. He highlighted the advancements in virus reduction capacity and the critical role of classical virology in ensuring safety. His insights underscored the importance of continuous vigilance and adaptation in pathogen safety and surveillance.

Impact of U.S. Policy Changes

Steven Grossman analyzed the challenges posed by new policies and budget cuts in key agencies such as the CDC, NIH, and FDA. He highlighted the breakdown in the sense of common good and the need for continuous vigilance and adaptation. Grossman's analysis provided a practical perspective on navigating the evolving regulatory environment.

Role of Advocacy and Strategic Adaptation

The speakers stressed the importance of advocacy in securing support for pathogen safety and surveillance programs. They discussed the need for strategic adaptation of research proposals to align with current priorities, emphasizing the importance of unmet medical needs in securing funding and support. Their insights underscored the need for continuous engagement with both the administration and Congress to ensure the continuation of vital programs.



WFH GLOBAL FORUM

AI Analysis

Bias and Gaps in Women's Care

Dr Angela Weyand
Associate Professor, Department of Pediatrics, University of Michigan

Dr Stephanie Seremetis
CVP and Chief Medical Officer for Rare Disease, Novo Nordisk

Dr Michelle Sholzberg
Division Head of Hematology Oncology, Medical Director of Coagulation Laboratory, Director of Hemequity Lab & Hematology-Oncology Clinical Research Group, St. Michael's Hospital
Dr Robert Sidonio
Pediatric Hematologist, Aflac Cancer and Blood Disorders Center

Moderator: Dawn Rotellini
BOD Lay member, World Federation of Hemophilia, Chief Operating Officer, National Bleeding Disorders Foundation

VOXO

Summary

The session delves into the biases and gaps in women's care, particularly focusing on bleeding disorders. The discussion highlights the challenges women face in diagnosis, representation in clinical trials, and the lived experiences of those with bleeding disorders. Dr. Angela Weyand opens the session by addressing sexism in diagnosing iron deficiency, emphasizing how menstruation is stigmatized and contributes to underdiagnosis. Dr. Weyand points out that iron deficiency is incredibly prevalent, affecting approximately 2 billion people worldwide, and disproportionately impacts women and children. The stigmatization of menstruation and the normalization of heavy periods within families often lead to iron deficiency being overlooked, despite its significant symptoms and health repercussions. The flawed reference ranges for hemoglobin and ferritin further perpetuate health inequities, leading to systemic underdiagnosis and lack of appropriate treatment for women with iron deficiency.

Dr. Stephanie Seremetis discusses the inclusion of women in clinical trials, especially those with hemophilia. She reveals that historically, women were excluded from these trials due to concerns about confounding results, but recent efforts have shown that their inclusion does not compromise the analysis of efficacy and safety. Dr. Seremetis emphasizes the necessity of designing inclusion criteria around symptomatology rather than gender and discusses how menorrhagia should be treated as a separate issue in trials. She presents early data showing that women in clinical trials behave similarly to men in terms of bleeding episodes, thus supporting their inclusion in future trials. The American Society of Hematology's efforts to ensure representation in clinical trials are also highlighted, emphasizing the importance of reflecting the epidemiology of the disease and the patient population.

Dr. Michelle Sholzberg provides insights into the lived experiences of women with bleeding disorders, using von Willebrand disease as an example. She illustrates how heavy menstrual bleeding significantly impacts the quality of life for women, often leading to iron deficiency and anxiety. Dr. Sholzberg's research reveals that many women normalize their heavy bleeding due to family experiences, resulting in diagnostic delays and misrecognition.

Dr. Sholzberg stresses the importance of systemic solutions, including harmonizing guidelines and improving healthcare provider awareness to prevent normalization and dismissal of symptoms. She also addresses the broader issue of heavy menstrual bleeding among all women of reproductive age, pointing out flaws in current assessment tools and the need for precise definitions and methodologies to quantify bleeding accurately.

Dr. Robert Sidonio discusses the impact of new nomenclature on care for women with hemophilia, emphasizing the need to better register and recognize these patients. He highlights the challenges in including women in registries and clinical trials and underscores the importance of personalized prophylaxis and factor replacement therapies. Dr. Sidonio points out that women with mild hemophilia often have similar bleeding symptoms to men, including mucocutaneous and joint bleeds, and stresses the need for hormonal therapies as cost-effective treatment options. He calls for more research and specificity in dosing guidelines, as well as better documentation and tracking of bleeding symptoms and treatments to improve care for women with hemophilia.

The session concludes with a discussion on the importance of normalizing conversations about menstruation and vaginal bleeding to improve care for women and girls with bleeding disorders. The speakers emphasize the need for healthcare providers to be aware of and comfortable discussing these topics with patients, as well as the importance of including women in clinical trials and research to ensure their experiences are accurately represented. The session calls for systemic changes to address the biases and gaps in women's care, including better education, awareness, and guidelines to support women with bleeding disorders.

Overall, the session provides a comprehensive overview of the challenges faced by women with bleeding disorders and offers recommendations for improving diagnosis, treatment, and representation in clinical trials. The speakers highlight the importance of addressing systemic biases, improving healthcare provider awareness, and ensuring that women are accurately represented in research and clinical practice. The session underscores the need for continued efforts to close the gaps in care for women with bleeding disorders and to ensure that their voices and experiences are fully recognized and valued.

Bias and Gaps in Women's Care
Thursday, November 13, 2025

Takeaways

Biases in Diagnosing Iron Deficiency

Angela Weyand highlights the significant impact of sexism and stigmatization of menstruation on the underdiagnosis of iron deficiency in women. The normalization of heavy periods within families and flawed reference ranges contribute to systemic health inequities. Addressing these biases is crucial to improving diagnosis and treatment for women with iron deficiency.

Inclusion of Women in Clinical Trials

Stephanie Seremetis emphasizes the importance of including women in clinical trials, revealing that their exclusion historically stemmed from concerns about confounding results. Recent data shows that women behave similarly to men in terms of bleeding episodes, supporting their inclusion in future trials. Designing inclusion criteria around symptomatology rather than gender is essential for accurate representation.

Lived Experiences and Systemic Solutions

Michelle Sholzberg and Robert Sidonio discuss the lived experiences of women with bleeding disorders and the impact of new nomenclature on care. Sholzberg stresses the need for systemic solutions, improved healthcare provider awareness, and harmonized guidelines to prevent normalization and dismissal of symptoms. Sidonio highlights the importance of personalized prophylaxis, factor replacement therapies, and better documentation to improve care for women with hemophilia.

WFH GLOBAL FORUM

AI Analysis

von Willebrand Disease

Dr. Nathan Connell
Chief of Medicine, Brigham and Women's Faulkner Hospital

Dr. Emna Gouider
Hematologist, Aziza Othmana Hospital- University Tunis El Mana

Elizabeth Clearfield
Senior Research Manager, Institute for Policy Advancement Limited

Dr. David Lillicrap
Professor, Queen's University

Moderator: Tatiana Bathfield
BOD Lay member, World Federation of Hemophilia Founding member / Secretary, Haemophilia Association of Mauritius

VOXO

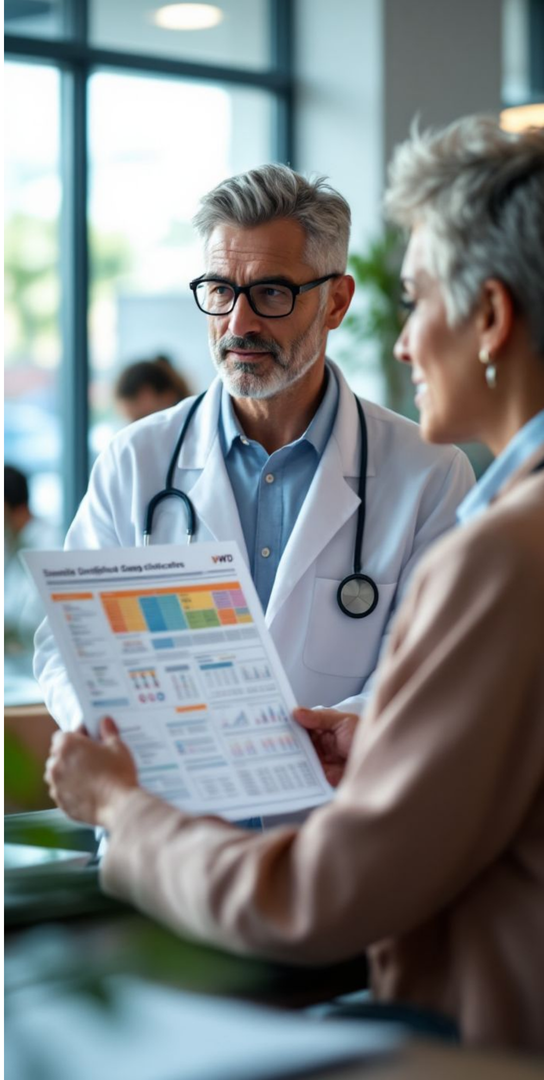
von Willebrand Disease
Thursday, November 13, 2025

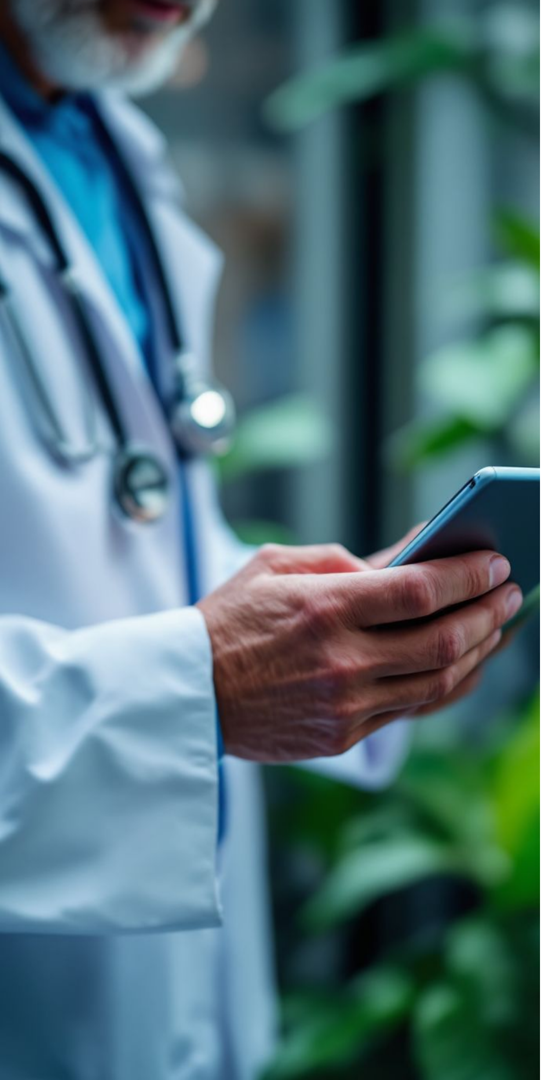
Summary

The session on von Willebrand Disease (VWD) brought together experts from various fields to discuss advancements in diagnosis, treatment, and the overall impact of VWD on patient lives. The discussion highlighted the complexities of diagnosing VWD, the importance of accurate prevalence data, the need for effective prophylactic treatments, and the development of new therapies that could significantly improve patient outcomes. Each speaker contributed insights from their respective areas of expertise, providing a comprehensive overview of current challenges and future directions in VWD management.

Dr. Nathan Connell opened the session by addressing the prevalence and access to prophylaxis in VWD post-guidelines. He emphasized the difficulties in estimating the prevalence of VWD due to variations in definitions, diagnostic methods, and geographic regions. Connell highlighted the importance of international guidelines in providing clarity but noted that implementation remains challenging, especially in low- and middle-income countries. He discussed the benefits of prophylaxis for patients with severe bleeding phenotypes and stressed the need for broader access to these treatments, citing the World Federation of Hemophilia's data on the low use of prophylaxis in type 3 VWD patients.

Professor Emna Gouider from Tunisia focused on simplifying the diagnosis of VWD, particularly in low- and middle-income countries. She argued that while comprehensive testing is ideal, it is often impractical in resource-limited settings. Gouider suggested prioritizing a few key assays, such as VWF activity, antigen, and factor VIII levels, to diagnose VWD effectively. She emphasized the need for qualified lab staff and raised concerns about the underdiagnosis of VWD, especially in women. Her recommendations aimed at improving diagnosis and advocating for better care and access to treatment.





Elizabeth Clearfield discussed the Core Outcome Set project, Core VWD, which aims to establish a standardized set of outcomes for clinical trials in VWD. She highlighted the importance of measuring outcomes that are meaningful to patients, such as quality of life, bleeding frequency, and severity. Clearfield emphasized the need for consistent data collection across trials to facilitate comparisons and improve treatment access. She also addressed the gaps in existing quality of life instruments for VWD and outlined plans to adapt the PROBE instrument for better coverage of patient-reported outcomes.

Dr. David Lillicrap explored future treatments for VWD, presenting several novel therapies currently in development. He discussed repurposing existing agents like recombinant VWF and Emicizumab, as well as new treatments such as aptamers, monoclonal antibodies, and nanoparticles. Lillicrap highlighted the potential of these therapies to offer more effective and less burdensome options for patients. He stressed the importance of continued innovation and research to address the unmet needs of VWD patients, especially those with severe bleeding phenotypes.

The session concluded with a Q&A segment, where panelists addressed various questions from the audience. Topics included the impact of early prophylaxis on angiodysplasia, the risks of hormone treatments in young girls, and the importance of accurate diagnostic tools. The speakers reiterated the need for tailored guidelines and treatments that consider the unique challenges faced by patients in different regions. They also emphasized the importance of collaboration between clinicians, researchers, and patient advocates to improve outcomes for all individuals affected by VWD.

Overall, the session provided a detailed examination of the current state of VWD research and treatment, highlighting both the progress made and the challenges that remain. The discussions underscored the importance of accurate diagnosis, effective prophylaxis, and innovative therapies in improving the lives of VWD patients worldwide.

von Willebrand Disease
Thursday, November 13, 2025

Takeaways

Challenges in Estimating VWD Prevalence

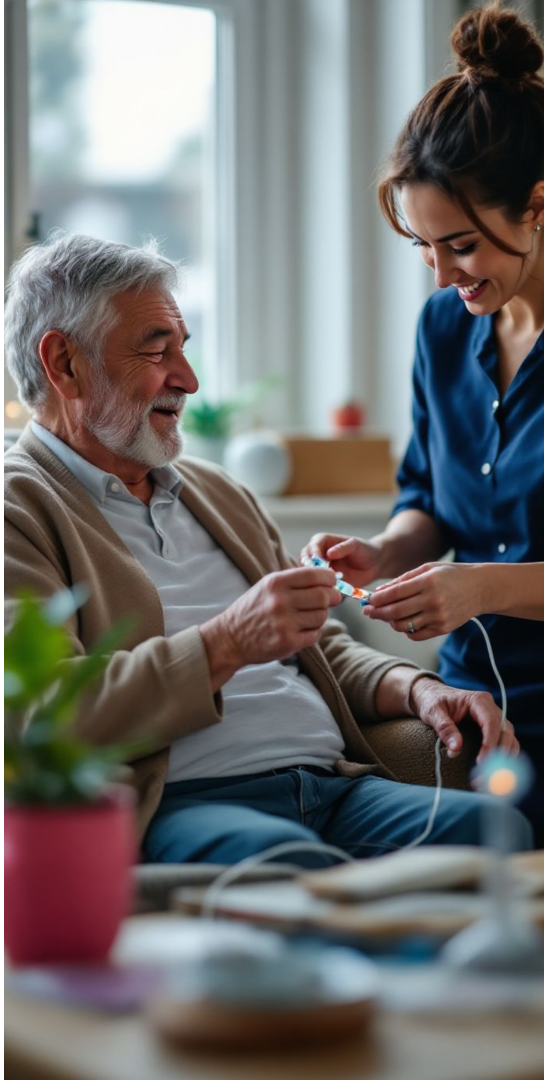
Dr. Nathan Connell emphasized the difficulty in accurately estimating the prevalence of von Willebrand Disease (VWD) due to variations in definitions, diagnostic methods, and geographic regions. He highlighted the importance of international guidelines in providing clarity but noted that implementation remains challenging, especially in low- and middle-income countries. Accurate prevalence data is crucial for advocating for better access to treatments and improving patient outcomes.

Simplifying VWD Diagnosis in Resource-Limited Settings

Professor Emna Gouider argued that comprehensive testing for VWD is often impractical in low- and middle-income countries. She suggested prioritizing a few key assays, such as VWF activity, antigen, and factor VIII levels, to diagnose VWD effectively. This approach aims to improve diagnosis rates, especially for underdiagnosed cases in women, and advocate for better care and access to treatment in resource-limited settings.

Innovative Therapies for VWD

Dr. David Lillicrap presented several novel therapies currently in development for VWD, including aptamers, monoclonal antibodies, and nanoparticles. These therapies have the potential to offer more effective and less burdensome treatment options for patients. Continued innovation and research are essential to address the unmet needs of VWD patients, particularly those with severe bleeding phenotypes, and improve their quality of life.



AI Analysis

Is the World Ready for Rebalancing Agents?

Dr. Johnny Mahlangu
Professor and Head of Department, University of the Witwatersrand and NHLS

Dr. Mike Makris
Professor, University of Sheffield

Dr. Silmara Montalvao
Scientific Director and Clinical Laboratory Scientist, University of Campinas-UNICAMP

Brian O'Mahony
CEO, Irish Haemophilia Society

Dr. Ekawat Suwantaroj
BOD Lay member; Vice President, World Federation of Hemophilia; Thai Hemophilia Patient Club

Dr. Maria Elisa Mancuso
Senior Hematology Consultant, IRCCS Humanitas Research Hospital

Moderator: Emna Gouider
Vice-President NMO, World Federation of Hemophilia / Chief, Hemophilia Center, Aziza Othmana Hospital

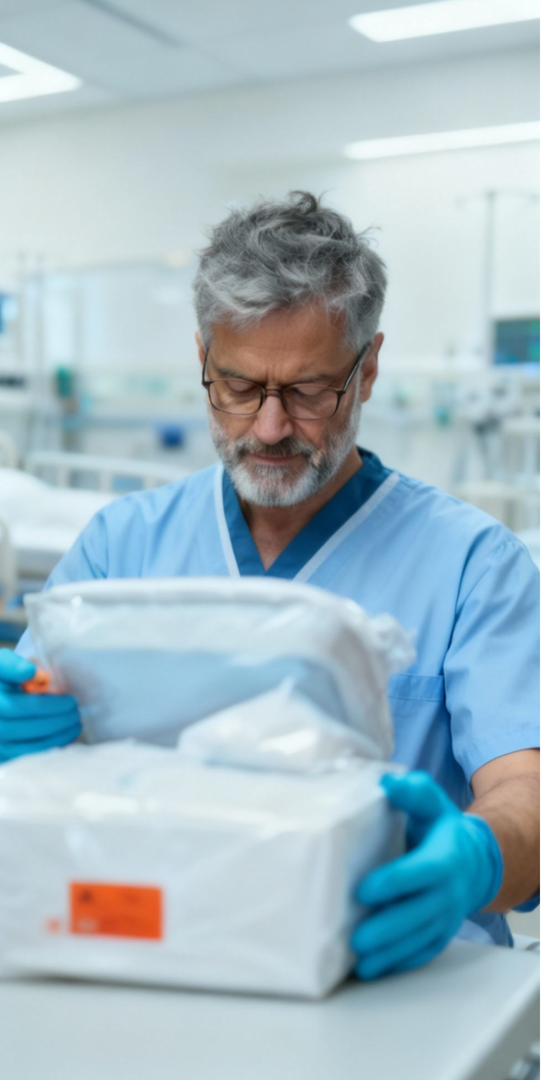
Summary

The session focused on the readiness of the world for rebalancing agents in hemophilia treatment, covering a wide array of topics including clinical efficacy, safety concerns, laboratory needs, procurement aspects, patient perspectives, and the potential use for other bleeding disorders. The speakers included prominent professors, CEOs, and practitioners who provided detailed insights into their respective areas of expertise.

Professor Johnny Mahlangu presented an overview of rebalancing agents, emphasizing their efficacy and practical implications. He highlighted the promising trial results for agents like Consisumab, Mistasemab, and Fitusiran, showcasing their ability to significantly reduce the mean annualized bleed rate and improve patient outcomes. He discussed the breakthrough bleed management guidelines, noting the importance of selecting the appropriate hemostatic agent and monitoring clinical and lab parameters. Mahlangu stressed the paradigm shift these agents represent in hemophilia management by offering sustained thrombin generation independent of factor replacement.

Professor Mike Makris addressed the safety of rebalancing agents, focusing on the thrombosis risks associated with these new therapies. He compared the rates of thrombosis in clinical trials to those observed with traditional treatments, emphasizing the need for careful monitoring and transparent reporting. Makris expressed concerns about the real-world application of these agents, particularly in older patients, and called for more detailed data on the risk of thrombosis, especially during major surgeries. He argued for individualized risk assessment and vigilance in using these therapies.

Dr. Silmara Montalvao discussed the laboratory needs and barriers for low-income and middle-income countries in implementing rebalancing agents. She highlighted the challenges of adapting laboratory routines to new monitoring requirements, such as measuring anti-thrombin levels and TFPI.



Dr. Montalvao emphasized the importance of quality and precision in assays, calling for better education and training for lab professionals. She noted the potential benefits of rebalancing agents in reducing treatment burdens but stressed the need for appropriate lab infrastructure.

Brian O'Mahony focused on procurement issues, noting the diverse range of prices for hemophilia treatments across different regions. He discussed the impact of new EU regulations and the potential for joint procurement initiatives to streamline processes and reduce costs. O'Mahony highlighted the complexity of gene therapy pricing models and the need for outcome-based agreements. He stressed the importance of realistic pricing and the potential for rebalancing agents to offer cost-effective alternatives for specific patient subgroups.

Dr. Ekawat Suwantaroj provided a patient perspective from Thailand, discussing the challenges faced by low-income families in accessing hemophilia treatments. He described the evolution of care in Thailand and the benefits of early treatment programs that allow home infusion of factor concentrates. Suwantaroj emphasized the need for patient education and engagement, noting the barriers to understanding and managing hemophilia effectively. He expressed hope for rebalancing agents to provide more convenient treatment options but stressed the importance of addressing language barriers and ensuring patients are well-informed.

Dr. Maria Elisa Mancuso explored the potential use of rebalancing agents for other bleeding disorders. She discussed the mechanistic differences and the need for further studies to determine their efficacy and safety in conditions like von Willebrand disease and rare bleeding disorders. Mancuso highlighted the complexity of coagulation pathways and the importance of individualized treatment approaches. She called for more research to optimize the use of these agents and ensure they are suitable for a broader range of patients.

Takeaways

Efficacy and Practical Implications of Rebalancing Agents

Rebalancing agents like Consisumab, Mistasemab, and Fitusiran show significant promise in reducing the mean annualized bleed rate and improving patient outcomes. They represent a major paradigm shift in hemophilia management by offering sustained thrombin generation independent of factor replacement, thereby addressing some unmet needs in traditional therapies.

Safety Concerns and Monitoring Needs

Professor Mike Makris highlighted the thrombosis risks associated with rebalancing agents, emphasizing the need for careful monitoring and transparent reporting. Individualized risk assessment is crucial, especially for older patients and those undergoing major surgeries. The real-world application requires vigilance and detailed data to ensure patient safety.

Laboratory and Procurement Challenges

Dr. Silmara Montalvao and Brian O'Mahony discussed the laboratory needs and procurement issues for implementing rebalancing agents in low-income and middle-income countries. Quality and precision in assays are essential, along with better education and training for lab professionals. Realistic pricing models and outcome-based agreements can help reduce costs and ensure broader access to these therapies.

AI Analysis

Availability of products

Dr. Patrick Robert
Independent Consultant, Marketing Research Bureau

Dr. Radek Kaczmarek
Assistant Research Professor of Pediatrics, Wells Center for Pediatric Research, Indiana University School of Medicine

Dr. Glenn Pierce
Vice-president Medical, World Federation of Hemophilia

Moderator: Brian O'Mahony
CEO, Irish Haemophilia Society, Ireland

Summary

The session delved into the availability of hemophilia products, focusing on the historical evolution, current landscape, and future directions in hemophilia treatment. Dr. Patrick Robert provided a historical overview, emphasizing the transition from plasma-derived factor products to recombinant and extended half-life products. He highlighted the growth in global usage and the impact of technological advancements on hemophilia care. Radek Kaczmarek discussed the current treatment landscape, noting the diversity of available therapies, including plasma-derived, recombinant, and gene therapies. He underscored the challenges and risks in developing new treatments, emphasizing the importance of reliable and durable therapies.

Dr. Glenn Pierce and Brian O'Mahony debated whether access to all products is necessary or sustainable for achieving treatment for all. Pierce argued that the ultimate goal should be zero bleeds for all patients, suggesting that conventional products should not be sent to low-income countries if they are not deemed effective enough in high-income countries. O'Mahony countered that while zero bleeds is an ideal goal, it is more practical to focus on incremental improvements and provide access to safe and effective treatments that can significantly enhance the quality of life for patients in developing countries.

The session also touched on the economic aspects of hemophilia treatment, with discussions on the varying costs of plasma-derived products and the impact of procurement systems on pricing. Speakers highlighted the importance of competition and diverse product availability in driving down costs and making treatments more accessible globally. There was recognition of the progress made through humanitarian aid programs and advocacy efforts, which have contributed to improved treatment access in many countries.

A significant part of the discussion revolved around the role of gene therapy and other advanced treatments. While gene therapy holds promise, there are concerns about its long-term efficacy, safety, and affordability. Speakers acknowledged that gene therapy might not be suitable for everyone and stressed the need for continued innovation to offer better protection against bleeding. The variability in gene therapy outcomes and the inability to re-administer due to neutralizing antibodies were noted as significant challenges.

The session concluded with reflections on the future directions in hemophilia treatment. There was consensus that innovation must continue to bridge the gap between current therapies and the ultimate goal of a hemophilia-free life.

The importance of setting ambitious goals while recognizing practical limitations was emphasized. Speakers called for coordinated efforts among national member organizations, governments, and advocacy groups to ensure better protection and access to advanced therapies for all people with hemophilia.

Overall, the session provided a comprehensive overview of the current state and future prospects of hemophilia treatment, highlighting the progress made and the challenges that remain. The importance of balancing ambitious goals with practical realities was a recurring theme, with a focus on ensuring equitable access to effective treatments globally.

Takeaways

Transition from Plasma-Derived to Recombinant Products

The session highlighted the historical shift from plasma-derived factor products to recombinant and extended half-life products. This transition has significantly improved hemophilia care, offering safer and more efficient treatment options. The availability of these advanced products has driven global growth in hemophilia treatment usage.

Debate on Treatment Goals

Glenn Pierce argued for the ultimate goal of zero bleeds for all patients, while Brian O'Mahony advocated for incremental improvements. Pierce emphasized that conventional products should not be sent to low-income countries if they are not effective enough in high-income countries. O'Mahony countered that practical access to safe and effective treatments can significantly enhance quality of life for patients in developing countries.

Challenges of Gene Therapy

Gene therapy holds promise for hemophilia treatment but faces challenges such as long-term efficacy, safety, and affordability. The variability in gene therapy outcomes and the inability to re-administer due to neutralizing antibodies are significant hurdles. Continued innovation is necessary to offer more reliable and durable therapies that can better protect against bleeding.

WFH GLOBAL FORUM

AI Analysis

Keynote

Dr. Cedric Hermans
Head, Hemophilia Center, Cliniques Universitaires Saint-Luc

Moderator: Dr. Mike Makris
Professor, University of Sheffield, United Kingdom

VOXO

Keynote
Friday, November 14, 2025

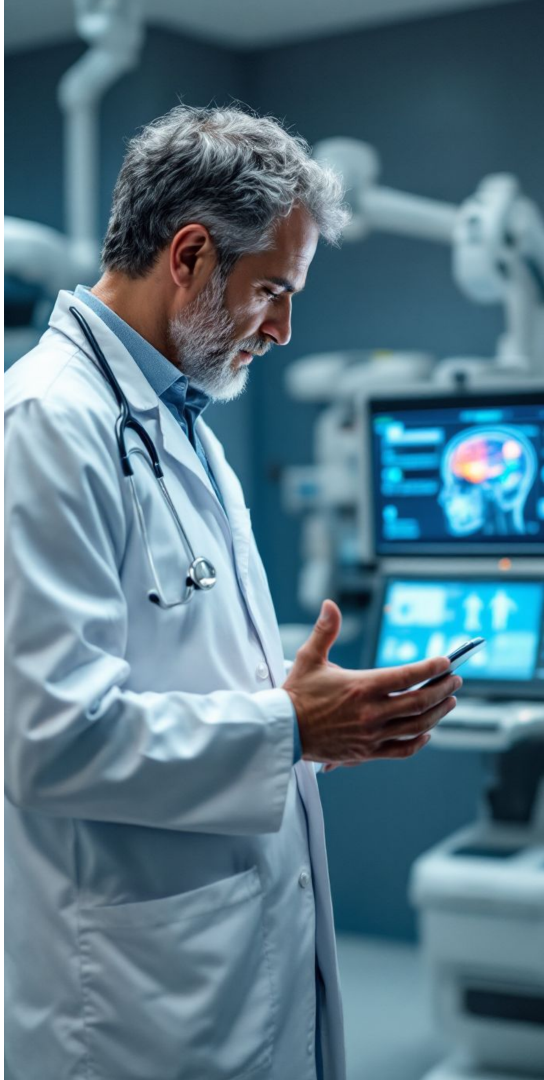
Summary

The session delved into the multifaceted impact of artificial intelligence (AI) on hemophilia care, led by Dr. Cedric Hermans, Head of Hemophilia Center at Cliniques Universitaires Saint-Luc, and moderated by Dr. Mike Makris, Professor at the University of Sheffield. Dr. Cedric Hermans initiated the discussion by highlighting current ambitions in hemophilia treatment, including diagnosis for all patients, minimal treatment burden, zero bleeds, safety, and normal life, and posed the question of whether AI could make these goals achievable. He provided an overview of AI's foundational concepts, emphasizing its ability to learn from data, recognize patterns, and analyze large amounts of information, yet noted its lack of consciousness and emotional understanding.

Dr. Hermans shared his personal use of AI tools like ChatGPT for professional tasks, underscoring AI's utility in summarizing complex papers, supporting differential diagnoses, and generating new content efficiently. He acknowledged AI's strengths in object recognition, predicting outcomes, and content generation, while cautioning against overestimating its understanding and intelligence, as AI operates on statistical probabilities rather than genuine comprehension.

Dr. Hermans explored the current landscape of AI in hemophilia, citing a limited number of publications on the topic but noting its potential in improving detection of joint damage, predicting bleeding risks, and understanding blood coagulation complexities. He argued that AI could enhance personalized medicine, drug discovery, and patient engagement but recognized significant limitations due to the limited amount of high-quality data available and the interpretability of AI models. Dr. Hermans stressed the need for rigorous validation and critical considerations regarding data privacy, security, and algorithmic bias. He highlighted AI's potential to improve patient education and empowerment through chatbots, which can provide personalized responses and support patients in managing their condition. This could be particularly valuable in regions with limited access to healthcare professionals, offering a scalable solution to patient education and engagement.

The discussion then shifted to AI's role in transforming the patient-doctor relationship, suggesting that AI could become an integral part of interactions, providing support in shared decision-making and enhancing patient empowerment. Dr. Hermans emphasized the importance of accessible, high-quality, and trustworthy data for AI to function effectively.



Keynote
Friday, November 14, 2025

Dr. Hermans described ongoing efforts in Brussels to develop AI-driven chatbots tailored to hemophilia patients, illustrating the potential for AI to collect vast amounts of data and provide personalized learning experiences. He also addressed the challenges of ensuring the accuracy and reliability of AI-generated information, advocating for restricted access to validated sources and the importance of transparency in AI's operations.

The session further examined AI's impact on medical education, with Dr. Hermans noting the transformative effect of AI tools like ChatGPT on teaching methodologies and student engagement. He expressed concerns about the future of traditional education models, as AI's capabilities continue to grow, and encouraged the integration of AI into educational systems to enhance learning experiences. Dr. Hermans advocated for generative engine optimization, a practice of shaping content for AI systems to find, interpret, and reuse, stressing the need for trustworthy, clear, and well-structured information accessible to AI. He proposed that medical journals and publications should adapt to ensure their content is easily usable by AI, facilitating the dissemination of knowledge.

Dr. Hermans also discussed AI's role in clinical practice, highlighting its utility in patient follow-up, data retrieval, decision-making, and record-keeping. He described the implementation of AI tools in hospitals, such as the EPIC system, which integrates patient information across various domains, enabling efficient data management and holistic patient care. Hermans shared examples from his hospital, where AI has been employed to provide summaries of patient data and support clinical decisions. He underscored the potential of AI to optimize health systems at multiple levels, from individual clinics to national healthcare infrastructures.

Finally, the session considered AI's potential in therapeutic innovation, particularly in gene therapy for hemophilia. Dr. Hermans outlined various strategies suggested by AI, including predicting optimal AAV capsids, identifying ideal candidates, and detecting hepatotoxicity. He emphasized the need for robust data collection and follow-up on treated patients to improve future gene therapy approaches. Dr. Hermans argued that AI could help design better clinical trials, integrate data, and enhance post-marketing surveillance. He concluded by advocating for the integration of AI into research efforts to detect gaps in knowledge and focus on areas most in need of innovation, while maintaining a human contribution to ensure creativity and originality in scientific endeavors.

Keynote
Friday, November 14, 2025

Takeaways

AI's Potential in Hemophilia Care

AI has the potential to revolutionize hemophilia care by achieving ambitious treatment goals, improving personalized medicine, and enhancing patient education and empowerment. However, this requires high-quality data, transparency, and rigorous validation to address AI's limitations. AI-driven tools, such as chatbots, can provide personalized responses and support, especially in regions with limited access to healthcare professionals.

Transforming Medical Education and Clinical Practice

AI is transforming medical education and clinical practice, optimizing patient follow-up, decision-making, and health system management. Integrating AI into educational systems can enhance learning experiences, while adapting publications for AI usability can facilitate knowledge dissemination. AI tools, like EPIC, enable efficient data management and holistic patient care, demonstrating the extensive potential of AI in healthcare.

AI in Therapeutic Innovation

AI can significantly contribute to therapeutic innovation, particularly in gene therapy for hemophilia, by predicting optimal AAV capsids, identifying ideal candidates, and detecting hepatotoxicity. Robust data collection and follow-up on treated patients are essential for improving future approaches. Integrating AI into research can help detect knowledge gaps and drive innovation, while maintaining a human contribution for creativity and originality.

WFH GLOBAL FORUM

AI Analysis

WFH Programs

Donna Coffin
Director, Data & Research, WFH

Dr. Assad Haffar
Director, Medical & Humanitarian Aid, WFH

Salome Mekhuzla
Director, Global Development, WFH

Moderator: Alain Baumann
CEO, World Federation of Hemophilia

Moderator: Marko Marinic
BOD Lay member, World Federation of Hemophilia / President, Croatian Haemophilia Society

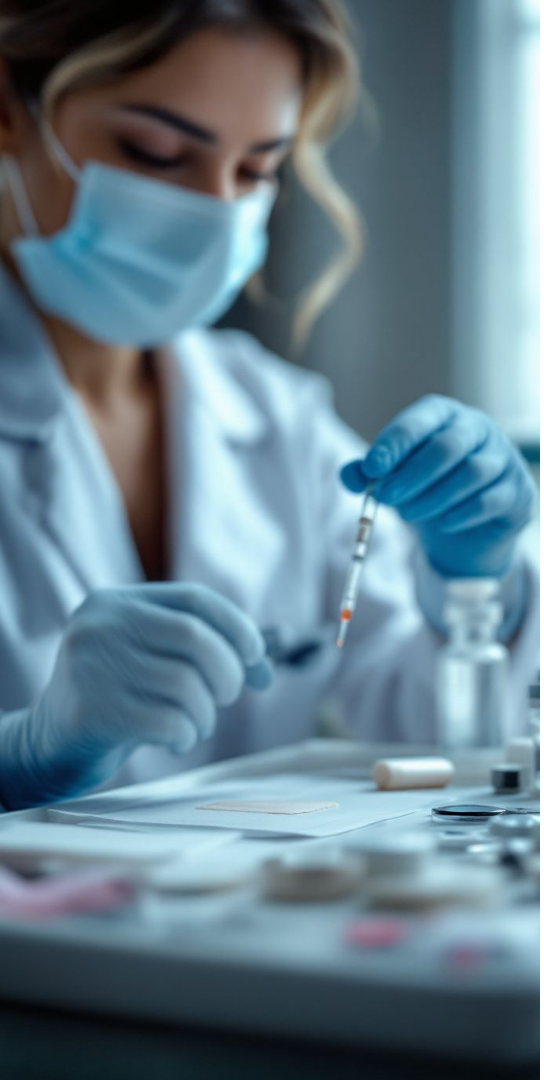
VOXO

Summary

The session provided an in-depth look into the World Federation of Hemophilia's (WFH) various programs and their impact, particularly focusing on the humanitarian aid, clinical and healthcare outcomes, advocacy efforts, and shared decision-making tools. Donna Coffin began by discussing the impact of WFH programs in Pakistan, focusing on their retrospective, observational study evaluating the humanitarian aid, PACT program, training, and education interventions. She highlighted the significant increase in the diagnosis of hemophilia patients, the uptake of prophylaxis, and improvement in the quality of life and reduction in hospitalizations among patients receiving treatment. These findings emphasize the positive impact of WFH programs on healthcare systems and patient outcomes in Pakistan.

Dr. Assad Haffar then presented on the sustainability of humanitarian aid and long-term impacts. He discussed the expanded humanitarian aid program that began in 2014, leading to substantial donations of factor concentrates and non-factor replacement therapies. Dr. Haffar emphasized the need for government involvement in purchasing treatment products and improving hemophilia care infrastructure. He provided examples from Egypt, Nepal, Bangladesh, and Pakistan where advocacy and demonstration projects led to increased government support and procurement of hemophilia treatments. Dr. Haffar highlighted the importance of demonstrating positive patient outcomes to convince governments to invest more in hemophilia care.

Salome Mekhuzla spoke about the milestone revision of the WHO essential medicines list. She explained the significance of the list and the WFH's efforts to update it to better reflect safe and efficacious treatments for hemophilia and von Willebrand disease. Mekhuzla detailed the comprehensive strategy employed by WFH, including publishing statements, engaging with the WHO, mobilizing global support, and submitting applications for changes. The successful outcomes included the addition of recombinant factor concentrates and emicizumab to the core list, and the removal of cryoprecipitate as a recommended treatment for hemophilia and von Willebrand disease.



Donna Coffin returned to discuss shared decision-making best practices. She described the development and implementation of the WFH shared decision-making tool, which aims to empower patients and healthcare providers in making informed treatment choices. Coffin outlined the consensus-based best practices identified during workshops in Western and Central Europe, emphasizing the importance of providing SDM tools in local languages, involving patients as equal partners, integrating SDM into regular clinical visits, and tailoring information to individual patient needs. These practices aim to improve patient engagement and healthcare outcomes through collaborative decision-making.

The session concluded with a brief discussion on the ethical considerations of limited treatment availability and the need for evidence-based advocacy to improve access to care. Dr. Haffar and Coffin addressed concerns about the ethical implications of rotating patients off prophylaxis due to limited resources, emphasizing the importance of prioritizing treatment for young children and those with severe conditions. They highlighted the need for more data on patient outcomes after discontinuation of prophylaxis to guide resource allocation decisions.

Overall, the session underscored the WFH's multifaceted approach to improving hemophilia care globally, through data-driven evaluations, sustained humanitarian aid, strategic advocacy, and empowering shared decision-making. The speakers emphasized the importance of collaboration, evidence-based practices, and engaging governments and communities to ensure sustainable and equitable access to hemophilia treatments.

WFH Programs
Friday, November 14, 2025

Takeaways

Impact of WFH Programs in Pakistan

Donna Coffin's presentation on the WFH programs in Pakistan showcased the positive effects of humanitarian aid, PACT program, and training interventions on healthcare outcomes. Significant improvements in diagnosis, prophylaxis uptake, quality of life, and reduction in hospitalizations were observed, highlighting the efficacy of WFH's multifaceted approach.

Sustainability of Humanitarian Aid

Dr. Assad Haffar discussed the expanded humanitarian aid program and its long-term impacts on hemophilia care. He emphasized the need for government involvement in purchasing treatment products and improving care infrastructure, using examples from Egypt, Nepal, Bangladesh, and Pakistan to illustrate successful advocacy efforts.

Revision of WHO Essential Medicines List

Salome Mekhuzla detailed the WFH's efforts to update the WHO essential medicines list, successfully advocating for the inclusion of recombinant factor concentrates and emicizumab, and the removal of cryoprecipitate for hemophilia treatment. The comprehensive strategy employed included publishing statements, engaging with WHO, and mobilizing global support to achieve these outcomes.

WFH GLOBAL FORUM

AI Analysis

Gene therapy and new technology access

Dr. Ben Samelson-Jones
Assistant Professor, Division of Hematology, Children's Hospital of Philadelphia

Mark Skinner
President and CEO, Institute for Policy Advancement Ltd

Dr. Renchi Yang
Head of Thrombosis and Hemostasis Centre, Institute of Hematology and Blood Diseases Hospital

Dr. Alok Srivastava
Head, Haematology Research Unit, St. John's Research Institute

Dr. Glenn Pierce
Vice-president Medical, World Federation of Hemophilia

Moderator: Radek Kaczmarek
Chair, WFH Coagulation Product Safety, Supply, and Acccess (CPSSA) Committee

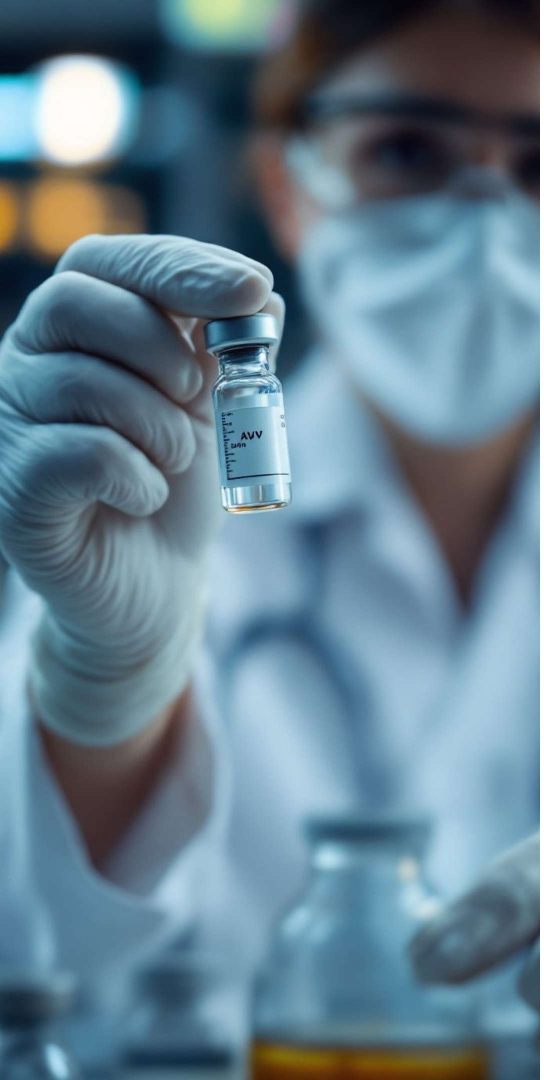
VOXO

Summary

The session on gene therapy and new technology access featured discussions from several prominent figures in the field, focusing on the current state, challenges, and future directions of gene therapy for hemophilia. Dr. Ben Samelson-Jones provided an overview of the progress in gene therapy as of 2025, highlighting the approved products for hemophilia B and A and discussing their uptake and ongoing dosing studies. He emphasized the transformative potential of gene therapy despite its limitations, such as variability in factor levels and the inability to redose. He also discussed the promise of new strategies to improve durability and allow pediatric dosing, highlighting ongoing studies and the importance of real-world data to better understand the therapy's efficacy and safety.

Mark Skinner addressed the broader implications of gene therapy within the context of the dream for a cure. He highlighted the challenges posed by discontinuation of programs and the slow uptake among patients. Skinner argued that the dream for a cure is complex and requires a clear definition, pointing out the need for transformative therapies rather than a complete cure. He discussed the impact of negative news from companies on venture capital investments and emphasized the importance of patient-centric data to drive decision-making. Skinner also raised concerns about the high costs of gene therapies and the barriers they create for patient access, calling for more thoughtful policy and pricing solutions.

Dr. Renchi Yang provided insights into the developments in China regarding gene therapy and advanced therapies for hemophilia. He highlighted the approval of several China-made products and ongoing clinical trials aimed at achieving self-sufficiency. Yang discussed the promising results from these trials, including stable factor levels and effective prophylactic use of immunosuppressants. He underscored the challenges related to pricing and accessibility in China, noting that despite lower costs compared to Western products, gene therapy remains expensive for Chinese patients.



Dr. Alok Srivastava focused on the prospects for self-sufficiency in India. He discussed the need for owning technology, manufacturing abilities, and regulatory processes to make gene therapy accessible and affordable. Srivastava shared updates on a novel gene therapy approach using stem cell-based methods, emphasizing its safety and efficacy. He highlighted the importance of reducing toxicity through innovative conditioning methods and presented data showing sustained factor levels and zero bleeds in treated patients. Srivastava also touched upon the broader implications for global access, noting the large number of patients in developing countries who could benefit from gene therapy.

Dr. Glenn Pierce discussed the safety issues surrounding gene editing as a form of gene therapy. He explained the basics of gene editing and its potential to provide permanent gene correction by inserting new genes directly into chromosomes. Pierce highlighted the challenges and safety concerns associated with gene editing, such as liver toxicity and off-target effects. He compared the safety profiles of gene editing and AAV gene therapy, noting improvements in AAV manufacturing and the potential benefits of gene editing in achieving lifelong production of clotting factors. Pierce concluded by emphasizing the need for careful assessment of risks and benefits and the importance of shared decision-making in advancing gene editing technologies.

The session concluded with a discussion on the financial viability of gene therapy and the role of venture capital in supporting innovation. Speakers highlighted the need for new business models that balance profitability with patient access and the importance of clear communication about the benefits and challenges of gene therapy. They discussed the impact of macroeconomic trends, such as the shift in venture capital investment towards artificial intelligence, on the funding landscape for gene therapy and called for continued advocacy and collaboration to ensure the sustainable development of new therapies.

Takeaways

Transformative Potential of Gene Therapy

Gene therapy has shown significant transformative potential for hemophilia, offering the possibility of durable factor levels and reduced bleeding episodes. Despite its limitations, such as variability in factor levels and the inability to redose, ongoing studies and new strategies are being developed to improve the therapy's efficacy and safety. Real-world data is crucial for understanding the long-term benefits and addressing the therapy's limitations.

Financial Viability and Policy Challenges

The high costs of gene therapies pose significant barriers to patient access, requiring thoughtful policy and pricing solutions. The impact of negative news from companies on venture capital investments highlights the need for clear communication about the benefits and challenges of gene therapy. New business models that balance profitability with patient access are essential for the sustainable development of new therapies.

Safety and Efficacy of Gene Editing

Gene editing offers the potential for permanent gene correction by inserting new genes directly into chromosomes, providing lifelong production of clotting factors. However, it comes with safety concerns such as liver toxicity and off-target effects, requiring careful assessment of risks and benefits. Improvements in AAV manufacturing and innovative conditioning methods are crucial for advancing gene editing technologies and ensuring their safety and efficacy.