Welcome to FH Europe Foundation’s January 2024 edition of Heart Beat.

FH Europe Foundation
The European Familial Hyperlipidaemias Foundation

Heart Beat News

FH Europe Foundation January 2024 Heart Beat News

Welcome to the first Heart Beat News of 2024!

We start the new year with kick-off meetings for two major and innovative projects dedicated to FH and precision medicine and personalised prevention - PerMed FH project and PERFECTO.

Building on this dynamic start, we are in full preparations for the Rare Disease Day, taking place on the rarest day of the year - February 29. On this occasion we are delighted to invite you to the Europe Rare Disease Summit. Coming up very soon also the Lp(a) Awareness Day. Read how you can collaborate and get involved in these important awareness campaigns!

This issue is full of updates on high-level events, initiatives and milestones from our network members as well as opportunities to get involved as a patient expert. Read about news from the industry, new treatments and many must-read publications and more.

Finally, don't overlook the spotlight on the PaRIS survey and the Awareness Days and Events Calendar 2024 prepared for you!

Wishing you an enlightening read and a delightful weekend ahead!

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FH EUROPE FOUNDATION NEWS

PERFECTO

A landmark initiative for personalized prevention, tackling an often-overlooked genetic cause of cardiovascular disease through the lens of social innovation, was launched in Warsaw with an official kick-off meeting on January 29 and 30.

PERFECTO stands for “**P**reventing th**E** P**R**eventable – **F**amilial Hyp**E**r**C**holesterolaemia paedia**T**ric screening for cardi**O**vascular Health.".

PERFECTO's mission is to generate evidence for the implementation of FH Paediatric Screening across Europe, which presents an unquestionable opportunity to make a significant positive impact for affected individuals, their relatives, and the wider society, as well as healthcare systems, which are facing a critical point post-pandemic.

Together with FH patients, advocates and ambassadors, clinicians, scientists, policymakers, and health systems experts, Partners embark on a journey to bring innovative solutions to the forefront of CVD prevention. This multidisciplinary collaboration underscores our commitment to empowering communities and ensuring a healthier future for generations to come.

PERFECTO is funded by the European Union. The views and opinions expressed are, however, those of the authors only and do not necessarily reflect those of the European Union or HaDEA. Neither the European Union nor the granting authority can be held responsible for them.

Read more about the project here.

PerMedFH Kick-off meeting in Lisbon

In a significant leap forward for the diagnosis and treatment of Familial Hypercholesterolemia (FH), the National Institute of Health Doutor Ricardo Jorge (INSA) recently hosted a pivotal launch meeting for the European project "PerMed FH – Personalizing diagnosis and treatment for Familial Hypercholesterolemia" on January 9 and 10 in Lisbon.

Led by INSA researcher and FH Europe Foundation's advisor Prof. Mafalda Bourbon, the project aims to revolutionise FH care through the development of personalised medicine tools, involving collaboration with esteemed institutions such as the University of Helsinki, the University Hospital of Rotterdam, the University of La Réunion Medical School, (France), and the FH Europe Foundation.

Recognized as one of the winners of the CaixaResearch Health Research Award 2023, "PerMed FH" secured one million euros in financial support from the "la Caixa" Foundation. The launch meeting served as a platform for project partners to present diverse work packages and discuss the three-year schedule. International collaborators also had the unique opportunity to tour the INSA laboratories, where crucial experimental work on functional studies related to genes associated with FH will be conducted.

Read more about it **here**.

Rare Disease Day

It’s less than one month until Rare Disease Day, which falls on the last day of February every year, and this year it's the rarest day, Thursday, February 29. The FH Europe Foundation advocates for the two rare familial hyperlipidaemias, Homozygous Familial Hypercholesterolaemia (HoFH) and Familial Chylomicronaemia Syndrome (FCS), which share many commonalities with the 6,000+ rare diseases currently identified. Rare Disease Day highlights the issues affecting the rare disease community. We advocate on this day in addition to the dedicated FCS Awareness Day held on the first Friday in November and the very first HoFH Awareness Day, which will be on May 4, 2024.

Read more about how you can support and collaborate **here**.

Driving Newborn Screening Innovations: Collaborative Efforts for Early Rare Diseases Detection

FH Europe Foundation is a member of the EURORDIS Newborn Screening Working Group (NBS-WG), which was set up to review current policy and practice in the field of NBS. Its goal is to develop principles for harmonious uptake/adoption of the NBS programs across Member States, aiming to deliver maximum benefit and improve outcomes for babies born with rare diseases based on its **11 Key Principles**. These principles were developed by EURORDIS, alongside its Council of National Alliances, Council of European Federations, and its members.

The NBS-WG works closely with Screen4Care, a 5-year European project funded under the IMI2 (Innovative Medicine Initiative) aimed at shortening the pathway to diagnosis using advanced technologies, including newborn screening and artificial intelligence applied to the diagnosis of rare diseases. In

January 2024, we submitted the genes causing both HoFH and FCS to be included in the panel for early detection. We will keep you informed as to the success of our submission.

In January 2024, genes associated with HoFH and FCS were submitted for inclusion in the NBS panel, marking a significant step in our ongoing efforts to improve early diagnosis.

Learn more about the EURORDIS Newborn Screening Working Group and our involvement **here**, and stay tuned for updates on the success of our submission.

Europe Rare Disease Summit 2024 - get your free ticket

Mark your calendars for the Europe Rare Disease Summit 2024, hosted by Bamberg Health on February 14 in Madrid. The events program covers topics of national government plans for rare diseases, access and financing, clinical research and innovation, artificial intelligence, and data. With over 45 speakers, including FH Europe Foundation's CEO, Magdalena Daccord, and Rare Diseases Project Manager, Jill Prawer, this summit aims to empower patients, foster cross-disciplinary collaboration, and influence policy.

Tailored for rare disease advocates, healthcare professionals, industry leaders, and more, the summit is a hub for knowledge exchange and networking.

The FH Europe Foundation is an official partner of the Summit and is pleased to offer complimentary tickets to rare disease patients and the FHEF Network Members, and others can attend at no cost with an invitation from the FH Europe Foundation. Don't miss this opportunity to be part of a pivotal dialogue shaping the future of healthcare.

Find more information here and to reserve your spot, click here.

Lp(a) 24 March
AWARENESS DAY

Lp(a) Awareness Day 24th March

In a little under two months FH Europe Foundation will be supporting Lp(a) Awareness Day for the third time. Established by the Family Heart Foundation in 2022, March 24th has become synonymous with raising awareness and educating people about Lp(a).

Everyone has Lp(a), but globally 1 in 5 of us have elevated levels, which is an independent risk factor for cardiovascular disease (CVD).

CVD is Europe’s biggest killer and costs the EU around €282 billion per annum. In December 2023 FH Europe Foundation travelled to Strasbourg to the EU Parliament and supported EACH (the European Association for Cardiovascular Health) in its mission to ensure that a Cardiovascular Health Plan is firmly on the agenda for the European People’s Party prior to the elections in June.

On March 21, FH Europe Foundation will be holding an interactive webinar titled “Ask Me Anything” to help develop knowledge and increase awareness about Lp(a). In the two weeks prior to this (7-20 March) you will be invited to email us with your questions about Lp(a), directed to one of our expert panellists. These questions will be answered live during the webinar.

To register for the webinar please click here. To email us your questions about Lp(a) please do so to askmeanything@fheurope.org.

Patient advocates voice represented in the Scientific Program Committee for 20th International Symposium on Atherosclerosis 2024

Year 2024 is the year when the International Atherosclerosis Society (IAS) is organizing its biennial symposium. The 20th International Symposium on Atherosclerosis is organised in collaboration with the Oman Society of Lipid and Atherosclerosis (OSLA). The event, themed "Toward Prevention of Cardiometabolic Diseases Through Precision Medicine," will take place from

December 4 to 6 in Muscat, Oman.

This invitation marks a remarkable stride in the field of cardio-metabolic diseases prevention, emphasizing the implementation of precision medicine and an active involvement of the patient community within the program committee.

The invitation follows our successful collaboration on the publication of the "International Atherosclerosis Society guidance for implementing best practice in the care of familial hypercholesterolaemia (FH)," available **here**.

Furthermore, this opportunity lays the foundation for our ongoing endeavours to support the creation of a patient advocacy group in the Middle East.

Read more about it **here**.

Get your Awareness Day and Events Calendar 2024

Embrace the excitement of the upcoming year with the FH Europe Foundation's Events Calendar for 2024! As we gear up for a thrilling year ahead, our dedicated team has prepared this calendar with easy colour-coded highlights, making it your go-to guide for the exciting events on our radar.

From **key awareness days and events** marked in red, signifying major initiatives and campaigns where your involvement is crucial, to **relevant awareness days and events** in dark blue, encouraging your engagement through likes, shares, and comments for a broader impact. Additionally, discover “***Dates worth knowing* about**” in the context of health and well-being, marked in light blue.

Visit our website to download the 2024 Events Calendar and be part of the action! Let's make 2024 a year of shared moments, impactful campaigns, and a healthier world. Join us on this exciting journey!

PATIENT
STORIES

OECD's PaRIS Initiative: Revolutionizing Patient-Centric Healthcare Globally

On the occasion of the **OECD - OCDE** High Level Policy Forum, which took place on January 22 in Paris, Health Ministers from different countries gathered to discuss the lessons learned from the COVID-19 crisis and the next health policy challenges.

During the event, panelists addressed the Patient-Reported Indicator Surveys (PaRIS), the first international survey assessing patients' experiences of primary care in 20 countries around the world.

As the voice of patients in Europe, the **European Patients' Forum** was part of the PaRIS Patient Advisory Panel, working to integrate essential patient needs into the broader initiative for establishing patient-focused healthcare systems.

The results of this initiative will be published in November 2024. Follow us for updates!

Watch a short clip about the survey **here**.
Read about the survey **here**.
Discover the event **here**.

Bulgaria - Federation Bulgarian Patients Forum

A high-level policy round table to address FH paediatric screening in the country!

A high-level policy round table is scheduled in Sofia at the Bulgarian Parliament on February 27. The meeting will be held under the auspices of the Health Commission. It builds on the relentless efforts of the national patient organization and the member of our network, the Federation Bulgarian Patients Forum, advocating for improvements in the FH screening program. The proposed new approach would see the age of children moved to 6-7 years, the introduction of (reverse) cascade screening, and the review of the reimbursement guidelines for PCSK9 inhibitors. The envisaged outcomes include a Memorandum signed by all participants endorsing the proposed changes and committing to their implementation.

The meeting will be attended by international speakers, including Prof Albert Wiegman, Prof. Urh Groselj, Dr Marius Geanta and Magdalena Daccord.

For more information, check our February newsletter.

Prepared by Federation Bulgarian Patients Forum

Ireland – Croí Ireland

Croí Engages Policy-Makers at Home and Abroad!

In December, representatives of Croí and HSVI were advocating for cardiovascular health on dual fronts, engaging with legislators at the **EU Parliament in Strasbourg** and the **Irish Parliament in Dublin**. As a member of the Global Heat Hub and as part of a European-wide delegation, Croí attended a special cardiovascular health event that took place at the European Parliament in Strasbourg. Croí & HSVI supported the European Alliance for Cardiovascular Health's (EACH) 'Vote Cardiovascular Health 2024' campaign, calling for a comprehensive EU.

First Croí Health Checks of 2024 - Many More to Come

Croí extends heartfelt gratitude to everyone who participated in the kickoff of Ireland's Lights Up Initiative at the Oran GAA Club in Co. Roscommon on Tuesday evening.

Caroline Dermody, Croí Community Nurse Lead, alongside our dedicated volunteer nurses Martina Beirne and Breda Walsh, provided essential health checks, including blood pressure and pulse. Taking charge of your heart health involves owning it, checking it, and addressing it.

Join them again at the Oran GAA Club on Tuesday, February 13, from 6.30 p.m. onwards for another opportunity to prioritize your well-being.

Read more about it **here**.

Prepared by Croí Ireland

Italy - AISC APS

AISC APS 2024 Initiatives: Elevating Cardiovascular Health Awareness

In 2024, AISC APS continues its impactful campaign, "Prevention of Cardiovascular Diseases and Knowledge of Heart Failure," reaching across Italian pharmacies and the AISC traveling clinic bus. With over 8 million Italians over the age of 65 facing chronic diseases, including cardiovascular issues, the organization aims to empower pharmacists as essential healthcare professionals.

Focusing on preventing cardiovascular diseases, recognizing risk factors, and addressing comorbidities like heart failure associated with diabetes and hypercholesterolemia, the campaign highlights the pivotal role of pharmacists. Specific attention will be given to the role of LDL cholesterol and Lp(a) as key risk factors for heart disease.

Additionally, AISC will develop content on Rare Disease Day (February 29th), shedding light on conditions like homozygous familial hypercholesterolaemia (HoFH) and Familial Chylomicronaemia Syndrome (FCS). The organization is committed to providing concrete tools for pharmacists to support citizens and intercept diseases in their early stages.

Prepared by AISC

UK - HEART UK

Heart UK's HoFH Networking Day - Birmingham, February 24, 2024

Heart UK is hosting an informal and informative networking day for individuals affected by Homozygous Familial Hypercholesterolemia (HoFH) on Saturday, February 24, 2024. The event will take place at Thinktank, Birmingham Science Museum, from approximately 10 a.m. to 4 p.m. Designed to foster community building, the day offers a relaxed setting for attendees to exchange knowledge, share experiences, and gain practical support. Families dealing with HoFH can expect an engaging and supportive environment during this event.

Read more about it here.

Prepared by Heart UK

GLOBAL HEART HUB

Global Heart Hub's Milestone: 100+ Supporters for the Global Cholesterol Action Plan in 2023

In a significant achievement, the Global Heart Hub's Global Cholesterol Action Plan garnered the support of over 100 signatories in 2023. This milestone builds momentum and backing for initiatives aimed at reducing the impact of unhealthy cholesterol levels on a global scale.

The Global Cholesterol Action Plan, a crucial component of GHH's Invisible Nation project, is designed to mitigate the effects of atherosclerotic cardiovascular disease (ASCVD), the leading cause of global mortality. Noteworthy supporters include the World Heart Federation, the European Atherosclerosis Society, the FH Europe Foundation, and patient organizations worldwide. The plan has received backing from 103 supporters spanning 41 countries.

These supporters will benefit from specially developed training materials, empowering them to drive positive change in the prevention and treatment of ASCVD in their respective countries. Global Heart Hub continues to lead the charge in the global fight against cardiovascular disease.

Read more about it here.

Prepared by Global Heart Hub

PARTNER NEWS

EURORDIS

Join EURORDIS at the European Conference on Rare Diseases and Orphan Products 2024

EURORDIS extends an invitation to the European Conference on Rare Diseases and Orphan Products (ECRD), recognized as the largest patient-led rare disease policy event in Europe. Scheduled for May 15 and 16, 2024, both online and at The Square in Brussels, the ECRD brings together patients, advocates, policymakers, healthcare professionals, and industry representatives to collectively shape future rare disease policies.

With over 1000 participants, the conference offers a unique opportunity for networking and knowledge exchange within the rare disease community. Collaborative discussions result in clear policy recommendations, influencing both EU and national policies.

Registration is currently open for ECRD 2024, providing 300 in-person spots with early-bird fees applicable until February 2. Additionally, submissions for poster abstracts are welcome until February 23. Patient groups, academics, and healthcare professionals engaged in rare disease research or public health projects are encouraged to participate.

Read more about it **here**.

EURORDIS launches the Social Policy Action Group (SPAG)

SPAG is a dedicated team of 20 volunteers from 13 European countries advocating for rare disease communities. Comprising individuals with rare diseases and family members, including experts in social policy and human rights, SPAG aims to shape policies promoting holistic care and safeguarding social and human rights.

Recruited through an open call, members like Jakub Gietka, founder of the Aiming for the Future Foundation in Poland, are committed to making a tangible impact. Jakub stated, "Through SPAG, I am committed to making a tangible difference, fostering awareness, and shaping a brighter future for the rare disease community."

SPAG's objectives include active participation in policy development, advocating for unmet needs, facilitating engagement in consultations, safeguarding social and human rights, raising awareness about the daily challenges faced by the rare disease community, and empowering individuals to advocate for holistic care and human rights.

Learn more about SPAG's mission and dedicated members **here**.

European Patients Forum (EPF)

CALL FOR INTEREST: JOIN THE HEU-EFS PATIENT ADVISORY GROUP

The European Patients Forum (EPF) and Global Heart Hub (GHH) are pleased to announce a call for representatives for a Patient Advisory Group (PAG) for the project ‘Harmonised Approach to Early Feasibility Studies for Medical Devices in the European Union (HEU-EFS)’. Early Feasibility Studies (EFS) are small-scale research studies or tests done in the very early stages of developing a medical device or treatment to see if it is practical, safe, and worth pursuing further. EFS help assess whether an idea or concept has potential before investing more time and resources into full-scale development and testing.

By increasing innovation in Europe, an overarching goal is for patients in Europe to have access to new, more innovative, safe, and effective medical devices.

Find out more about the project **on EPF’s website** and **on GHH website**. Also check out our **official project website**.

WORLD HEART FEDERATION (WHF)

From 22-27 January, WHF represented the global cardiovascular community at the 154th Executive Board of the **World Health Organization** in Geneva, Switzerland.

WHF Advocacy Committee Chair **Trevor Shilton** and the WHF Policy and Advocacy Team delivered targeted statements on key agenda items including Universal Health Coverage, Noncommunicable Diseases, Health Emergencies, Maternal and Child Health, Climate and Health, Well-being and Health Promotion, and others.

The goal was to ensure that cardiovascular disease remains a prominent topic in the agenda-setting conversations.

Learn more and read our statements **here**.

Empowering Lives: Implementing the WHF Roadmap for Cardiovascular Disease Prevention

The World Heart Federation (WHF) launched its updated Roadmap for Secondary Prevention of Cardiovascular Disease (CVD), recognizing the critical importance of follow-up care post-cardiac events. Annually, 20.5 million people succumb to CVD, responsible for nearly one-third of global deaths, with a majority attributed to atherosclerotic cardiovascular diseases (ASCVD). This umbrella term includes conditions like coronary artery disease and cerebrovascular disease, leading to premature death and substantial healthcare costs.

The updated Roadmap addresses barriers to effective secondary prevention strategies, acknowledging issues such as limited access to healthcare and medicines, clinical inertia, and insufficient infrastructure. Offering 15 core strategies, the Roadmap emphasizes solutions like ensuring affordable secondary prevention medications, improving access during hospital discharge and rehabilitation, and enhancing healthcare provider training for lifestyle management and medication adherence.

Surveying over 250 responses across 60 countries, WHF found disparities in secondary prevention tools, with 52% citing the lack of lifestyle intervention programs and 48% in low-income countries pointing to the scarcity of affordable medications. The Roadmap aims to guide practical tools and policy changes, ultimately enhancing the lives of 300 million people worldwide living with ASCVD and promoting cardiovascular health globally.

Read more about it **here**.

WHF's Vision: Cardiovascular Health for Everyone 2024-2026

The World Heart Federation (WHF) unveils its New Strategy 2024-2026, setting the course for "Cardiovascular Health for Everyone." Aligned with its mission, the strategy aims to become a trusted authority, champion health equity, and inspire global action. Recognizing that 80% of cardiovascular diseases (CVD) are preventable, WHF seeks to leverage collective wisdom for impactful change.

Central to the strategy is Vision 2030, which guides efforts to prioritize global cardiovascular health and acknowledges the intricate interplay between health behaviours, societal factors, and systemic challenges.

WHF commits to innovation, capacity building, and collaboration, utilizing technology and engaging a wider audience. This proactive approach aims to shape the current landscape, driving meaningful changes beyond the strategic cycle. WHF looks to the future with optimism, confident in its role as a convener, and the power of collective action to achieve cardiovascular health for all.

Read more about it **here**.

INDUSTRY
NEWS

Getting to the heart of the matter: improving cardiovascular health in Europe

The European Federation of Pharmaceutical Industries and Associations (EFPIA) is organizing a virtual event about the importance of having national cardiovascular plans that include secondary prevention measures.

The event will feature insights from top experts, including key researchers from the LSE study, offering an invaluable opportunity to understand the practical applications of this groundbreaking research.

Our very own Patient Ambassador and Community Engagement Manager, Emma Print will be speaking on the panel, along with Haseeb Ahmad, President of Novartis Europe; Panos Kanavos, Associate Professor of International Health Policy at the London School of Economics and Political Science; Dolors Montserrat, Member of the European Parliament, Group of the European People's Party, Spain and Tomislav Sokol, Member of the European Parliament, EPP Group, Croatia.

Recently published research by the London School of Economics (LSE), commissioned by the European Federation of Pharmaceutical Industries and Associations (EFPIA) Cardiovascular Health Platform, demonstrates that if even just 70% of people living with cardiovascular disease could have their risk factors better managed, 1.2 million deaths in the EU could be averted over the next ten years. For example, reducing LDL-cholesterol levels by just 1 mmol/L has been shown to reduce all-cause mortality by 10%.

Register and read more about it **here**.

NEW
PUBLICATIONS

International Atherosclerosis Society Roadmap for Familial Hypercholesterolaemia

Several clinical guidelines and calls to action have addressed the gaps in care for FH, but little attention has been devoted to implementation science and practice. The International Atherosclerosis Society (IAS) has developed evidence-informed guidance that provides a systematic compendium of clinical recommendations for the detection and management of patients with FH, supplemented with implementation strategies to optimize the deployment of models of care.

The guidance is divided into detection, management and implementation sections. Detection covers screening, diagnosis, genetic testing and counseling. Management covers ASCVD risk stratification, treatment of adults and children with heterozygous and homozygous FH, management of FH during pregnancy, and use of lipoprotein apheresis. Specific and general implementation strategies, guided by the Expert Recommendations for Implementing Change, are provided based on expert consensus.

Read the full publication **here**.

Recommendations of the Experts of the Polish Cardiac Society (PCS) and the Polish Lipid Association (PoLA) on the diagnosis and management of elevated lipoprotein(a) levels.

The first Polish recommendations for Lp(a) of the two main societies in the field - Polish Cardiac Society and Polish Lipids Association have just been published. They cover aspects of early measuring and diagnosis, as well as treatment in the absence of approved medication.

Read the full publication **here**.

LDL-C–Lowering Therapies for Adults and Children with Homozygous Familial Hypercholesterolemia: Challenges and Successes

Read the editorial piece in Circulation, a top journal of the American Heart Association, where Prof. Marina Cuchel and Prof. Raul Santos had the opportunity to comment on the current and future management options of HoFH in the context of the following two papers covering treatment options for children with HoFH.

Read the full publication **here**.

Rare Disease Moonshot: Europe’s Public-Private Coalition to Erase the Rare Disease “White Spots”

As we step into 2024, it's time to unveil the Rare Disease Moonshot and shed light on its purpose, participants, and objectives.

In a sneak peek, the paper concludes with a powerful message: "Together, we can improve the lives of people with a rare disease in Europe and beyond; make the European research ecosystem more attractive and efficient; and address the 'white spots' with increased speed and vigor. These are challenges that can only be solved by harnessing the collective intelligence of everyone engaged in this field."

For a comprehensive understanding, delve into our latest publication featured in the DIA Global Forum. Embrace and share the insights and tools crafted by the RD Moonshot, and join us in fostering collaborations and partnerships to propel this groundbreaking initiative forward.

Read more about it here.

TREATMENT NEWS

Novel treatment for HoFH available now for children in the UK

In the UK, the National Institute for Health and Care Excellence (NICE) issued a final draft guidance endorsing evinacumab (known under the commercial name Evkeeza) for NHS England. This marks a pivotal moment for homozygous familial hypercholesterolemia (HoFH) patients, providing an additional tool for managing the condition effectively. Evkeeza, the first ANGPTL3 inhibitor treatment, complements standard therapies and demonstrates potential clinical and economic benefits. Notably, it is recommended for use in children aged 12 and older, addressing a critical unmet need in this patient population.

Evinacumab has been approved in multiple countries, including the **U.S., Japan, Canada, Italy, Germany,** the **UK, and Wales**.

Read more about it here.

SAVE THE DATE

- **14 February -** Europe Rare Disease Summit, Madrid
- **29 February** - Rare Disease Day
- **24 March -** Lp(a) Awareness Day
- **4 May (NEW!) - HoFH Awareness Day**
- **24-26 May -** World Heart Summit 2024, Switzerland
- **8-10 November** – FHEF Annual Network Meeting 2024

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