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CHANGING PARADIGMS OF HEMOPHILIA CARE ACROSS LARGER SPECIALIZED TREATMENT CENTERS IN THE EUROPEAN REGION

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SELECTED HIGHLIGHTS

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FUNDING AND CONFLICT OF INTEREST

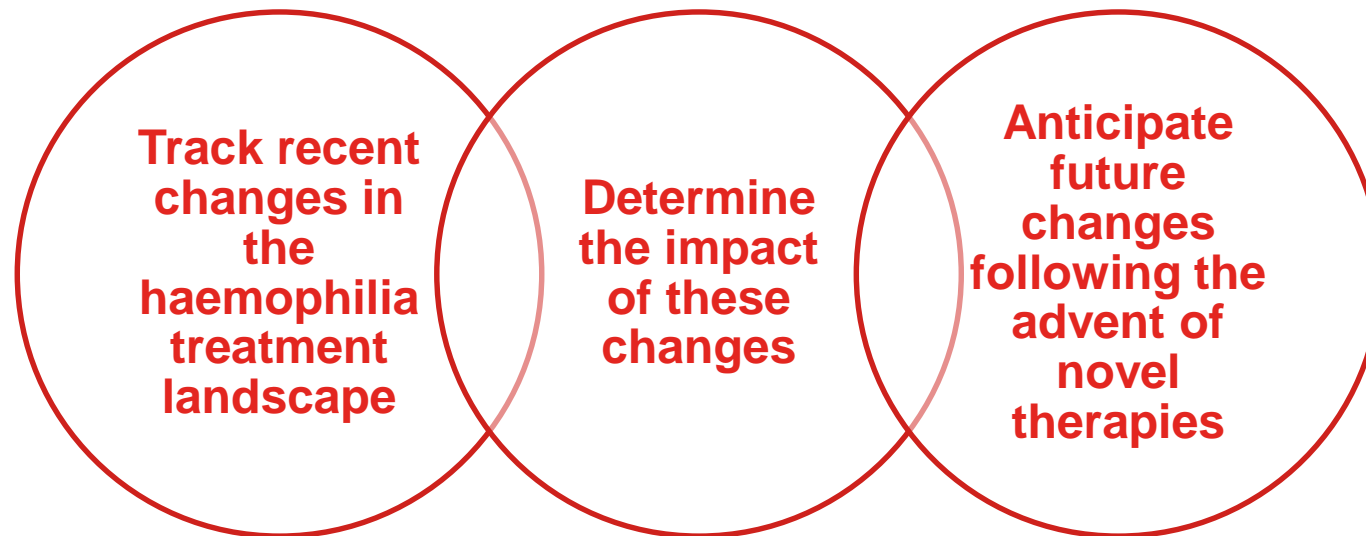
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- Early 2021, the **European Collaborative Haemophilia Network (ECHN)** conducted a survey to determine whether the paradigms of care have changed with the introduction of novel therapies for people with haemophilia¹⁻³
- A **survey** was conducted in 19 ECHN centres from 17 countries in the **European region**⁴
- The **aim** of the survey was to⁴:



RESULTS

PATIENT AND CENTRE DEMOGRAPHICS

- Centres treated a total of 4,710 people with **haemophilia A**, 1,067 people with **haemophilia B**, and 1,569 **carriers**^a
 - 1,792, 655, and 2,263 had mild, moderate, and severe haemophilia A
 - 417, 217, and 433 had mild, moderate, and severe haemophilia B
- 13 centres treated both **adults and children**
 - 4 centres treated adults only
 - 2 centres treated children only
- The most common **age** group being treated across both haemophilia A and B and across all disease severities was age 19-60 years



Countries included in the ECHN survey 2021

^a Categorized according to the most recent World Federation of Haemophilia (WFH) guidelines (Srivastava A, et al. Haemophilia. 2020;26 Suppl 6:1-158)
Windyga J, et al. Ther Adv Hematol. 2022. DOI: 10.1177/20406207221088462

RESULTS

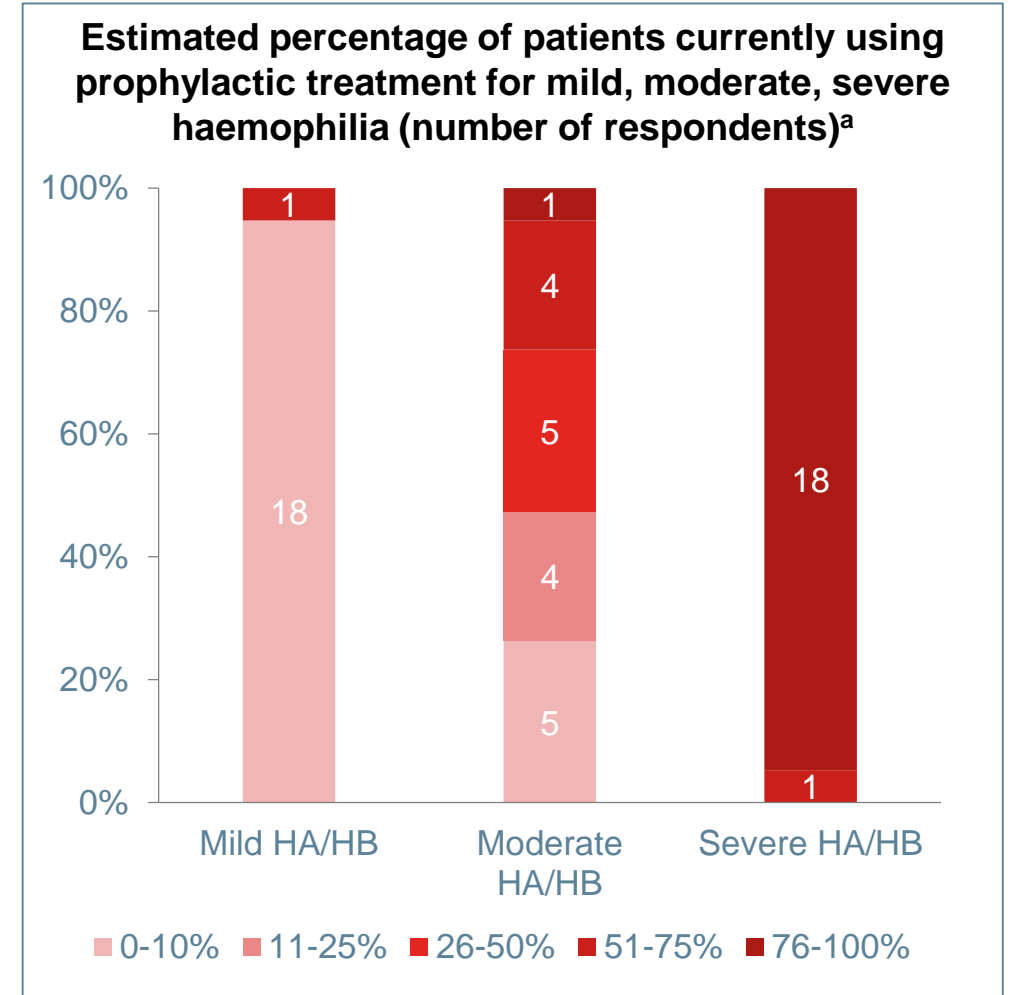
ORGANISATION, FUNDING, AND COLLABORATION

- Most centres (18/19) were **part of a university or teaching hospital**
 - All centres have at least one **accreditation**
 - Comprehensive care centre was most common designation (17 centres)
 - Centralised government **funding** was the most common source of funding (14/19 centres)
 - **Collaboration** between centres is commonplace (90%)
 - More than half of the centres share treatment protocols/guidelines
 - 18 centres participate in national **registries**, 11 in international registries
 - Cooperation with **patient organisations and industry** is commonplace (85%)
 - All centres have a strategy for **personalisation of treatment**
-

RESULTS

TREATMENT PATTERNS: PROPHYLAXIS

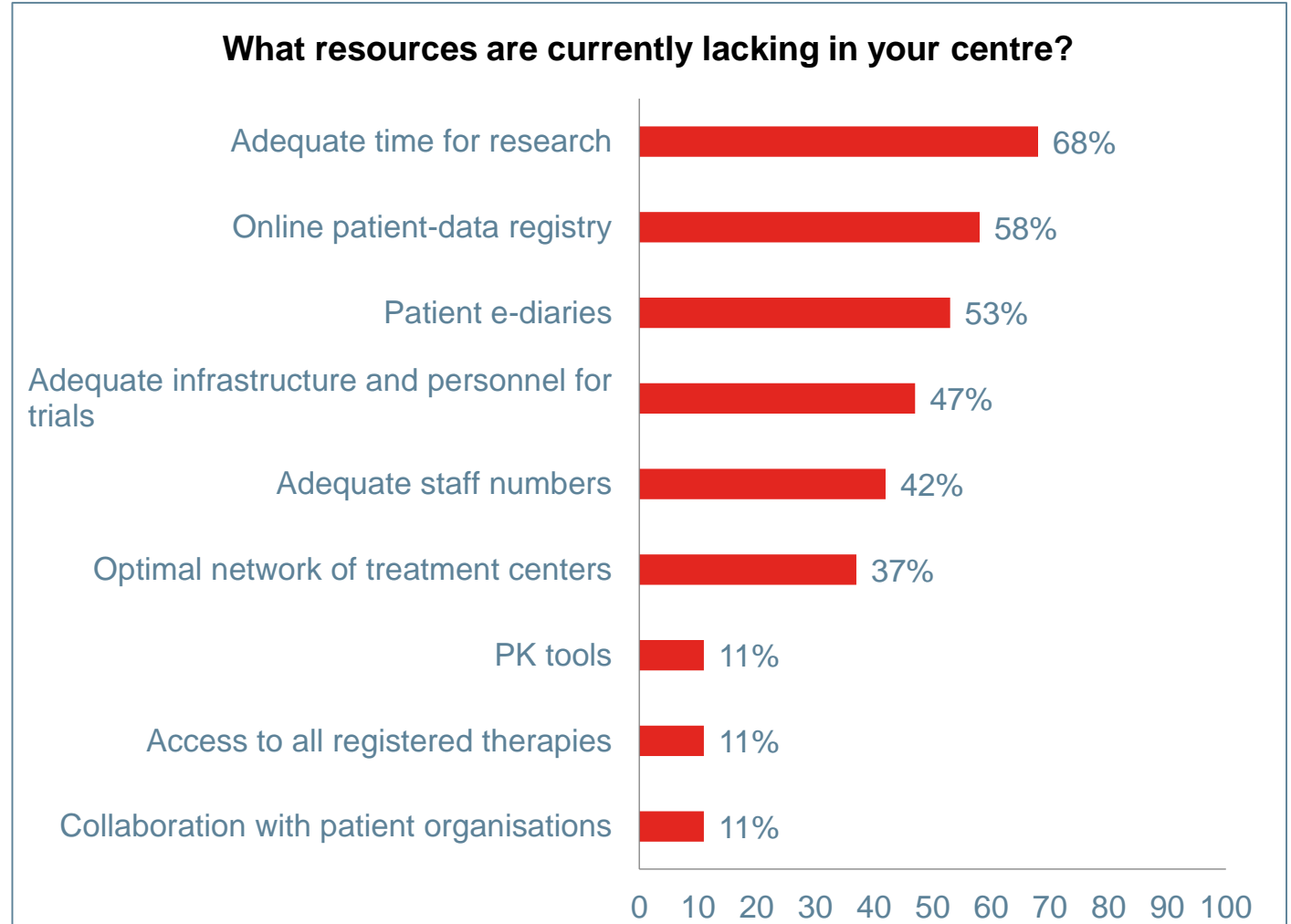
- **Most patients with severe haemophilia** were treated with prophylaxis
 - Only 5% of respondents reported reaching an **annualised bleeding rate of 0** in >76% of these patients
- Prophylaxis is **less common in mild and moderate** haemophilia
- Immune tolerance induction (**ITI is still a priority** in patients with inhibitors in most centres
 - ITI is commonly used alongside other therapies (e.g. emicizumab prophylaxis)
 - ITI use is guided by previous success of ITI, efficacy of current therapy, venous access, quality of life, and availability of alternative or combination therapies



RESULTS

CHALLENGES RELATED TO RESOURCING AND ORGANISATION (1)

- **Time limitations** related to research are a key concern
- Around one-third of centres report an optimal **network of centres** in their country as an ongoing concern
- More than half of centres indicate availability of **online patient-data registries** as an ongoing concern
- **Cost issues limiting access** to therapies is an ongoing concern
- **Clinical trial infrastructure** represents an ongoing concern

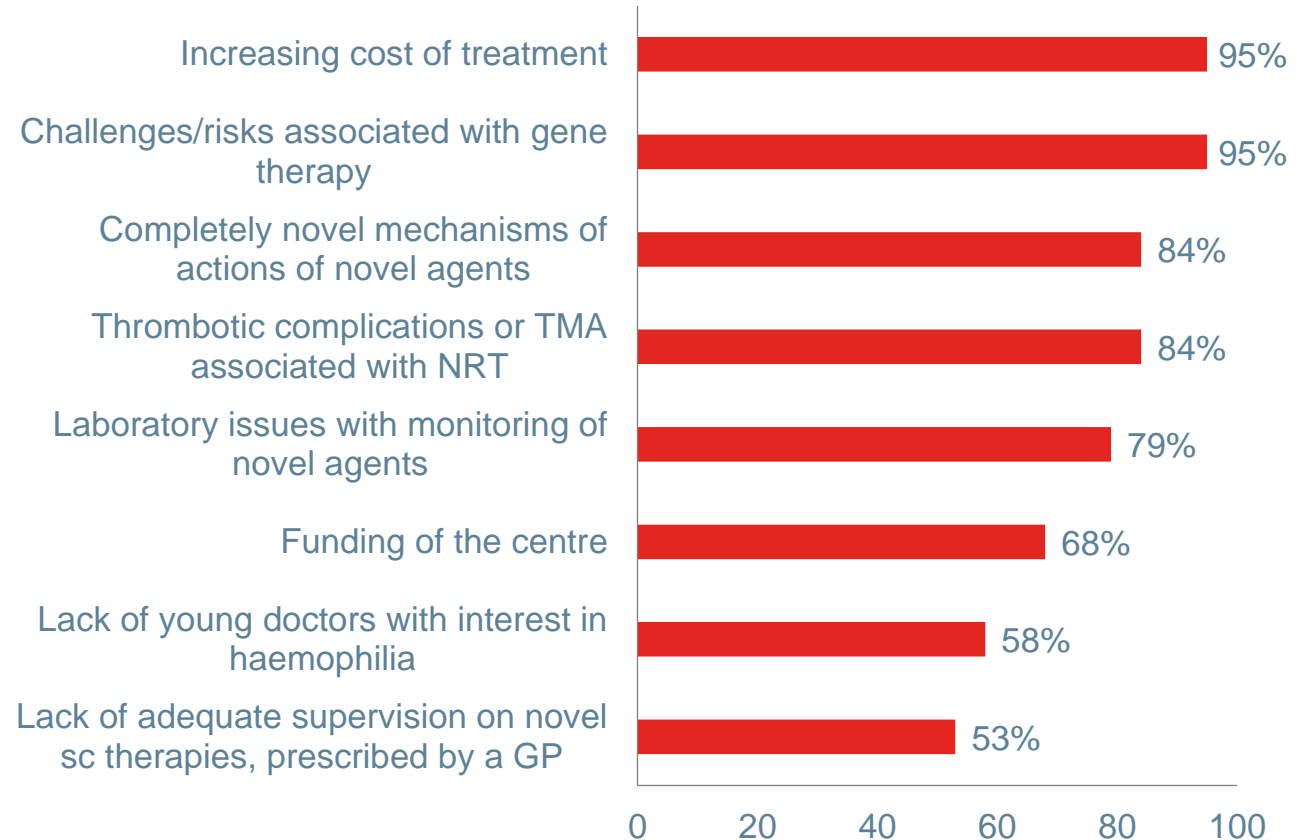


RESULTS

CHALLENGES RELATED TO RESOURCING AND ORGANISATION (2)

- Concern related to the **increasing cost of therapies** is near-universal
- There is ongoing concern related to the **increasing complexity of treatment and monitoring** requirements
 - Access to **skilled staff** is an area of concern
 - **Education and training** is an ongoing concern
- Although around half of centres participate in **gene therapy** trials, more than half of centres overall indicated they are **not ready for implementation outside of clinical trials** and concern related to risks/challenges overall was near-universal

What challenges do you see with the innovations mentioned in this questionnaire?



RESULTS

AVAILABILITY OF PRODUCTS BY LICENSING AND REIMBURSEMENT STATUS

- Availability of treatment options **varies across countries and centers** and, in the case of products that are not yet licensed, is limited to use in a clinical trial setting
- **Extended half-life products and non-factor replacement therapies** were the **most 'available'**, with unrestricted access in the highest number of centers (14/19 and 12/19 centers, respectively)
- **Non-factor replacement therapies and extended half-life products** were most commonly available **free of charge**, either as a standard therapy or as part of a clinical trial

RESULTS

Country	Non-factor replacement therapies	Extended half-life products	Non-replacement therapy	Gene therapy
Czech Republic	Unrestricted availability; reimbursed	Unrestricted availability; reimbursed	Not available	Not available
Spain	Unrestricted availability; reimbursed	Unrestricted availability; reimbursed	Unrestricted availability; reimbursed	Unrestricted availability; reimbursed
Slovenia	Unrestricted availability; reimbursed	Unrestricted availability; reimbursed	Not available	Not available
Ireland	Unrestricted availability; reimbursed	Unrestricted availability; reimbursed	Available in clinical trials only	Available in clinical trials only
Belgium	Unrestricted availability; reimbursed	Unrestricted availability; reimbursed	Not available	Available in clinical trials only
Norway	Limited availability; reimbursed ^a	Unrestricted availability; reimbursed	Not available	Available in clinical trials only
Austria	Unrestricted availability; reimbursed	Limited availability; reimbursed where available	Available in clinical trials only	Available in clinical trials only
Germany (3 centers)	Unrestricted availability; reimbursed	Unrestricted availability; reimbursed	Available in clinical trials only (n=1)	Available in clinical trials only (n=2)
			Not available (n=2)	Not available (n=1)
Croatia	Unrestricted availability; reimbursed	Limited availability; reimbursed where available	Available in clinical trials only	Not available
Poland	Limited availability; reimbursed where available ^a	Not available	Available in clinical trials only	Not available
Italy	Unrestricted availability; reimbursed	Unrestricted availability; reimbursed	Unrestricted availability; reimbursed	Available in clinical trials only
Slovakia	Limited availability; reimbursed where available ^a	Limited availability; reimbursed where available	Not available	Not available
Sweden	Limited availability; charges may apply ^a	Limited availability; reimbursed where available	Available in clinical trials only	Available in clinical trials only
Netherlands	Limited availability; reimbursed where available	Limited availability; reimbursed where available	Not available	Limited availability; reimbursed where available
Israel	Unrestricted availability; reimbursed	Limited availability; charges may apply	Available in clinical trials only	Available in clinical trials only
France	Unrestricted availability; reimbursed	Unrestricted availability; reimbursed	Available in clinical trials only	Available in clinical trials only
Turkey	Limited or no availability; no data on reimbursement available	Limited or no availability; no data on reimbursement available	Limited or no availability; no data on reimbursement available	Limited or no availability; no data on reimbursement available

^a For patients with haemophilia A with inhibitors only.

Haemophilia care across specialised centres European region in 2022

- We have started to see to **tangible benefits of improved protocols of care**
- Accreditation, collaboration, and personalisation of treatment is commonplace
- The use of **prophylaxis** is well-aligned across centres, and is near-universal in severe haemophilia
 - However, issues related to annualised bleeding rates persist
- **Access** to and availability of treatments is not universal across the region

Unmet needs and key challenges in haemophilia care 2022

- Key ongoing concerns include **costs and accessibility**, **time** limitations, and **human resources**
- Standards and protocols, as well as the centres themselves, will have to **continue to evolve** if they are to provide the highest level of care
- To meet this requirement, there is a **clear need for engaging, ongoing education programs** for healthcare professionals working in the field of haemophilia that can be adjusted to the changing landscape of haemophilia therapy and monitoring

CONCLUSIONS



In 2022, we have started to see to the **tangible benefits** of developments in protocols of care, driven by efforts of the haemophilia community over the past decade



Key challenges remain to resourcing and organisation, with cost/access issues, time limitations, and education and staffing being paramount



As innovation increases costs and drives complexity, finding adequate solutions that will allow **universal access to haemophilia treatment** might prove to be the greatest challenge of all

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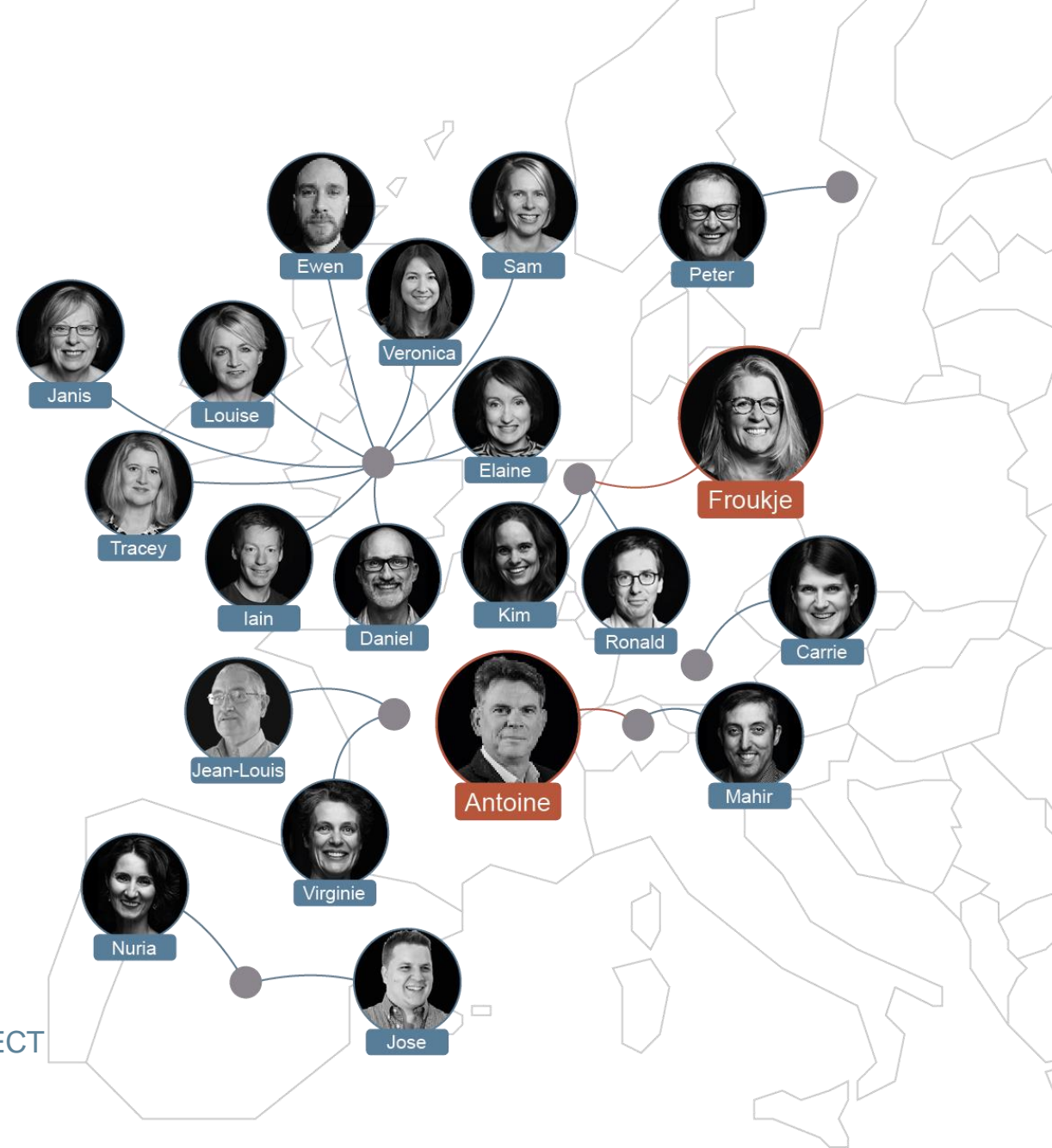
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