



The Dutch Hemophilia Registry HemoNED - Building an Ecosystem

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Disclosures for: Geertje Goedhart

Conflict	Disclosure - if conflict of interest exists
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Director, Officer, Employee	-
Shareholder	-
Honoraria	-
Advisory Committee	-
Consultant	-

Introduction

European Principles of
Haemophilia Care
(Fischer, Haemophilia 2008)

European Medicines Agency
*(McGettigan, Drug Safety
2019)*

National hemophilia registry essential for
quality of care and pharmacovigilance

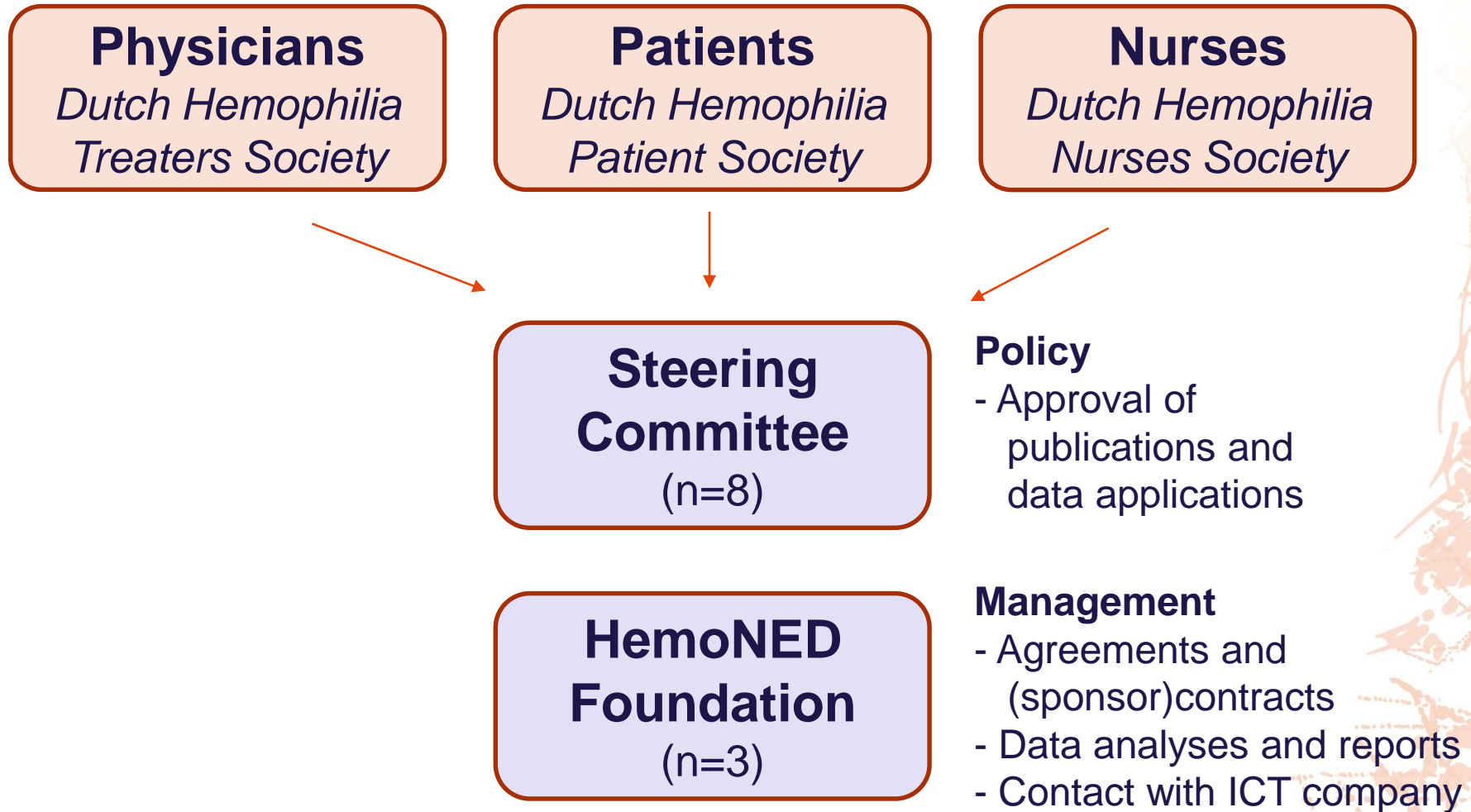
National initiative:
Control of registries for expensive medicines
*(National Health Care Institute & Ministry of
Health, 2019)*

Objectives

National hemophilia registry essential for:

- ❖ Numbers of patients by disorder and severity of disease
e.g., number and localization of severe patients
- ❖ Benchmarking
e.g., comparison of treatment between treatment centers
- ❖ International comparison
e.g. WHF Annual Global Surveys
- ❖ Pharmacovigilance & PMS studies
e.g., central reports to EUHASS
- ❖ Research
Prescribing trends
- ❖ Monitoring & evaluation of treatment
'VastePrik' app for patients and dashboard for health care providers

Methods: Organization



Methods: Support

Netherlands Organization for Health Research and Development

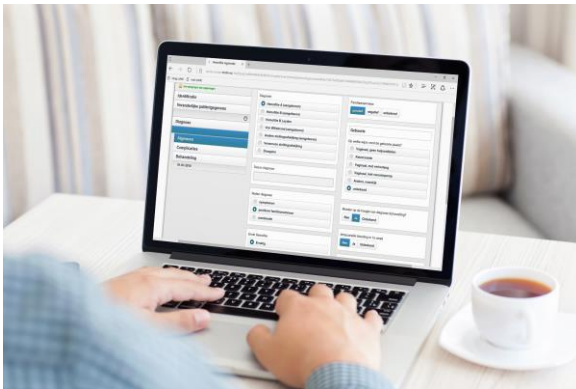
2-year grant to set up registry

Public-private partnerships

- Sponsor contracts with pharmaceutical companies
- No data access
- Annual general report and specific reports for pharmaceutical companies and hemophilia treatment centers
- Future
 - Annual pharmacovigilance reporting
 - PMS studies

Registry items

- **Patient data** *(for privacy reasons only visible for local treatment center)*
 - Name, patient number, birth date, gender etc.
- **Diagnosis, severity / type**
 - Hemophilia A / B (incl carriers)
 - Von Willebrand disease
 - Other factor deficiencies or platelet disorders
- **Inhibitor & viral infection**
 - Baseline: current, ever, never
- **Treatment plan**
 - Prophylaxis: product(s), dose & frequency
 - On demand: product(s)
- **Adverse events**
 - Inhibitors
 - Transfusion transmitted infections
 - Allergic reactions
 - Thrombosis
 - Deaths
 - Malignancies



Items are manually entered, pilot on automated import from electronic health records

'VastePrik' app & web page

Digital infusion log for patients on home treatment

- **Treatment**
 - Date/time, product, dose, batch number, reason
- **Bleeds**
 - Date/time, type, location, severity, cause
- **Extra**
 - Medication stock, barcode scanning, notifications, allergic reactions & joint pain

Online dashboard for patient and local center to monitor & evaluate treatment



Results: inclusion & demographics

Inclusion period:
Dec 2017 – Dec 2019

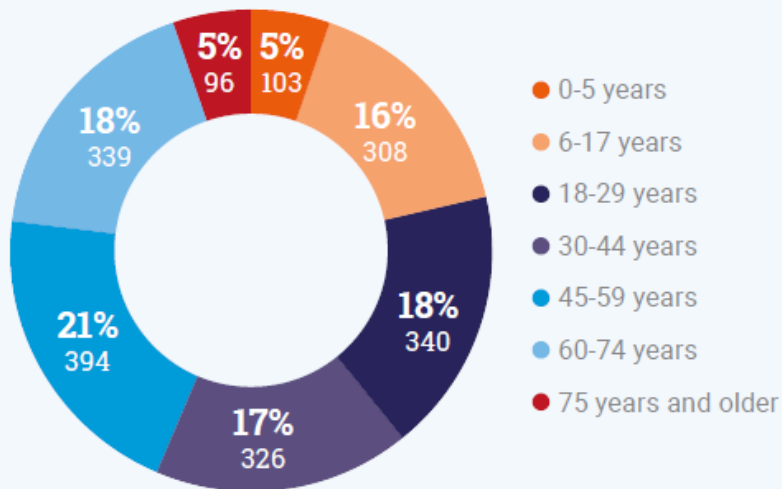
Total participants

Total completed **1907** (100%)

Age

Total completed **1906** (100%)

Missing **1**



Gender

Man **1529** (80%)

Woman **374** (20%)

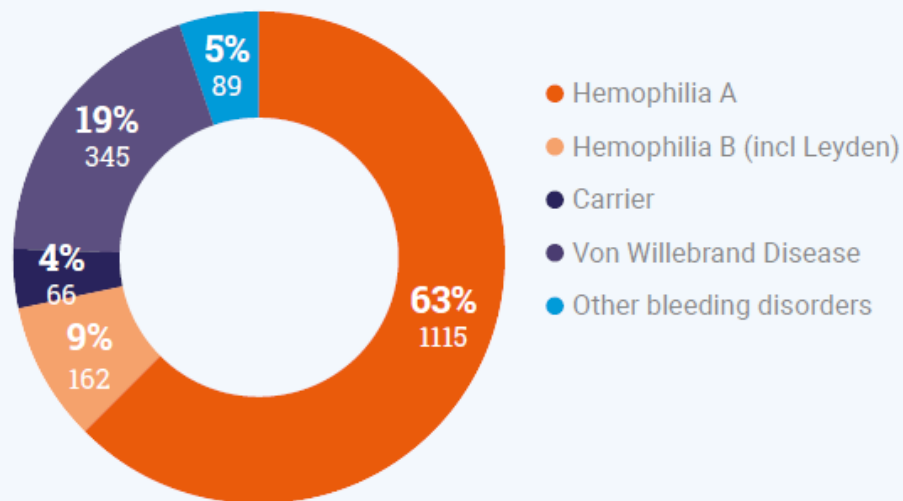
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Results: diagnoses registered

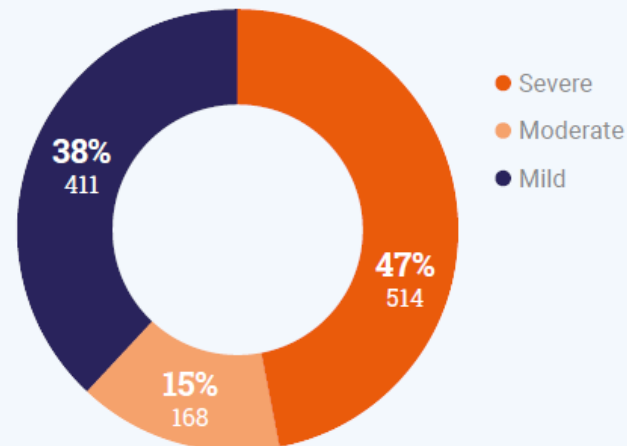
Diagnosis

Total completed **1777** (100%)

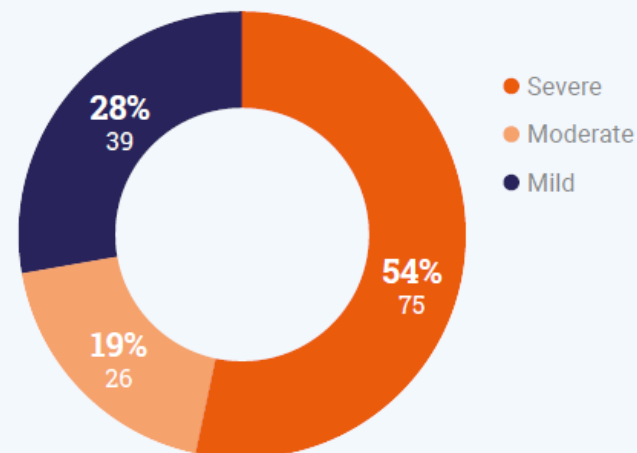
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Hemophilia A



Hemophilia B



Results: VastePrik app

VastePrik users (n=547) mainly severe hemophilia A / B

Infusions and bleeds

Table 11 Number of infusions by reason reported in VastePrik (2018-2019)

Reason infusion	Number of infusions	%
Prophylaxis	47851	88
Precaution (risky activities)	1372	3
(Directly following a) Bleed	2657	5
Aftercare (after a bleed or surgery)	2388	4
Total completed	54268	100

Table 12 Type of bleeds reported (2018-2019)

Type of bleed	Number of bleeds	%
Joint	1347	51
Muscle	528	20
Subcutaneous	185	7
Mucous membranes	138	5
Other	459	17
Total	2657	100

Table 13 Bleed severity and cause of reported bleeds (2018-2019)

Bleed severity	Number of bleeds	%
Low	714	27
Average	1468	55
High	475	18
Total	2657	100

Cause	Number of bleeds	%
Spontaneously	1158	44
Overload	620	23
Accident or trauma	478	18
Postoperative	24	1
Other	377	14
Total	2657	100

Discussion

- ✓ Dutch Hemophilia Registry successfully implemented in all Comprehensive Care Centres
 - Challenges: building organizational structure, lots of agreements, pushing the ICT company, privacy issues, finances, burden of manual registration, etc.

- ✓ 'VastePrik' digital infusion log used by severe patients
 - Challenges: bugs in mobile app, irregular use, registration errors, etc.

- ✓ From 1/1/2020 central registration of adverse events
 - Challenges: adverse events of not registered patients, data export from HemoNED to EUHASS

Future perspectives

- Automated import from electronic health records to HemoNED Registry to minimize manual registration
- Postmarketing surveillance for new treatments, e.g., Emicizumab, gene therapy
- Benchmarking, national & international
- Structural (financial) embedding of registry in Dutch governmental system of expensive medicines reviews

Presented on behalf of

Dutch Hemophilia patient society (NVHP)

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Thank you for your attention!

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