Theme 6: Global Rare Equity: Are we there yet?

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Global Access to Timely Accurate Diagnosis and Optimized Care

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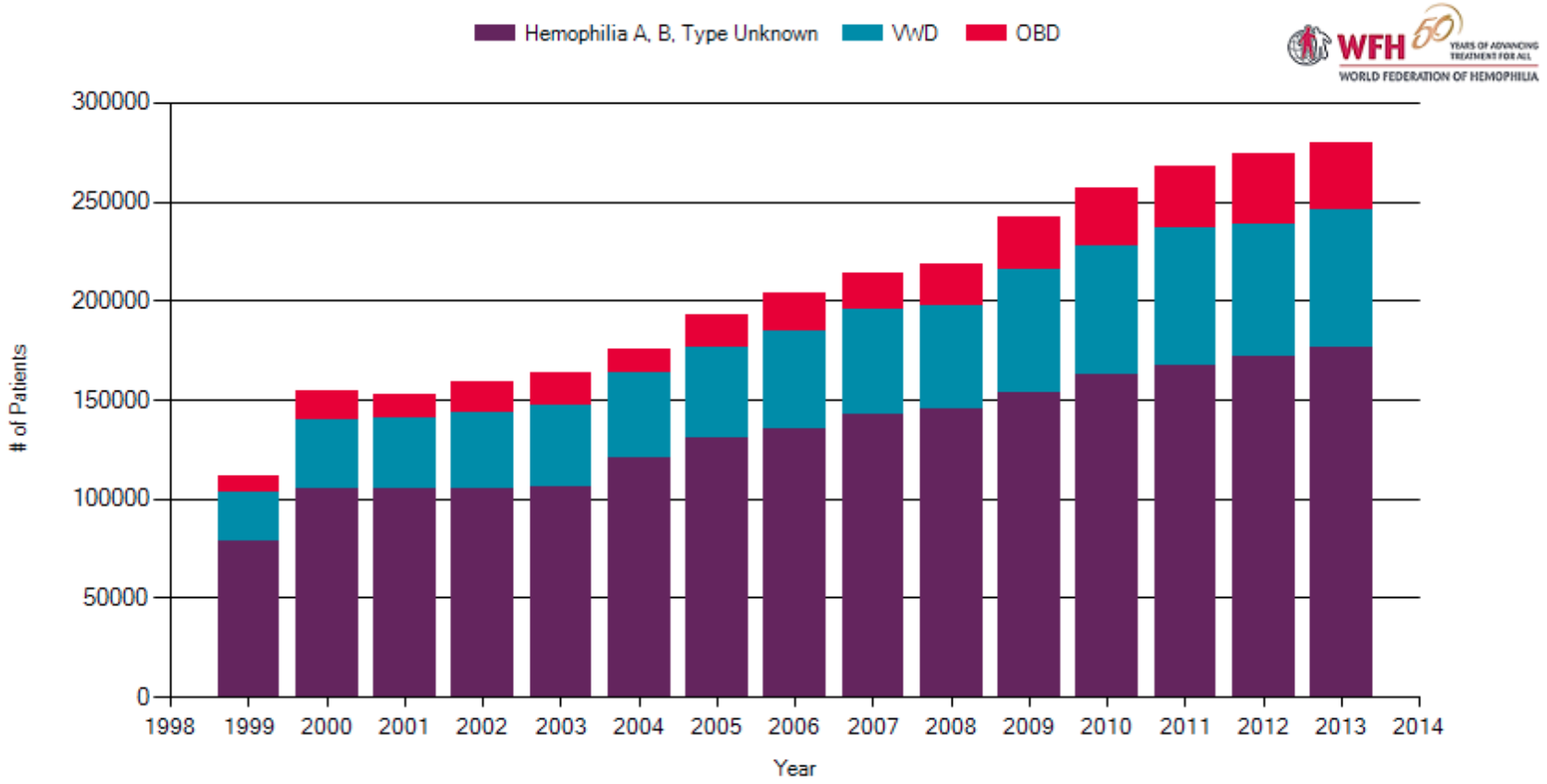
Focus on rare (plasma) diseases

- Plasma therapies heavily focused on rare diseases
- Diagnosis & treatment options overall increasing, but great majority of conditions have no treatment
- Policies & actions to support rare disease diagnosis & treatment are critical
- Differentiation for rare diseases & plasma therapies in reimbursement & regulation is needed
- Recognition & treatment of rare (plasma) diseases increasing globally

Increasing diagnosis
patients with bleeding disorders



Disclaimer: Not all of our members are able to report every year.
A list of participating countries and the last year they provided data can be found in the Report on the Annual Global Survey.

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Source: World Federation of Hemophilia Annual Global Survey 2013

Factor VIII use by country

IU per capita

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Source: World Federation of Hemophilia Annual Global Survey 2013

Plasma as a global strategic resource

- Changes in clinical practice in developed countries have reduced the need for red cells and significantly reduced volume of recovered plasma
- The need for plasma-protein therapies (PPTs) worldwide continues to increase
- Majority of plasma supplies for the manufacture of PPTs derives from the US - Geographic imbalance in plasma collection concerns that local disruptions of plasma supplies could affect regional and global availability of therapies
- Plasma collections should be increased outside the United States, including lower and middle income countries

Importance of international trade

- Conditions know no borders
- In a global environment, the ability to trade is critical
- Importance of intellectual property provisions for sustainable R&D environment
- More licensing opportunities reduce risk
- Clinical trials increasingly international

Proving Value is Part of the Access Solution

- Company responsibility to produce and share therapeutic value data for informed decisions
- Extensive company programs in place during stages of drug development and post-approval
- Support patient-centricity in clinical trial design and value measures
- Partner in advocacy around value measures and related access policies
- Access = diagnosis + treatment access

CSL Behring Programs

- Broad patient group support in conditions we treat:
  - Grants, aid-in-kind, partnering
  - Global, regional, national
    - WFH multi-year funding and product donations
    - IPOPI advocacy
    - ‘Alpha 1 Global’ capabilities and programs
    - HAE International
    - Regional umbrella groups
    - Multiple national and U.S. state groups

Working together, we can strengthen patients' access to medicines by:

- Enabling earlier diagnoses
- Providing faster, more efficient regulatory approvals
- Ensuring access to a range of treatment options
- Providing more medicines in lower and middle income countries

- More partnership, more global across all stakeholders
- Efforts to develop capabilities in the conditions we treat
- Creating global community
- Impacting policy, diagnosis, & treatment