

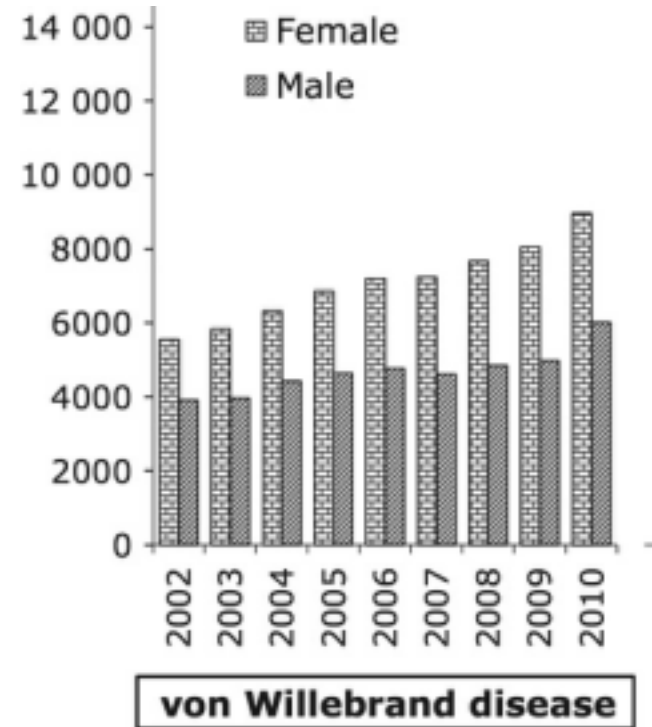
ATHN DATA SUMMIT 2016

Using the ATHNdataset to understand more about females with bleeding disorders

Kristina Haley, DO, MCR
The Hemophilia Center
Oregon Health & Science University

Females with Bleeding Disorders

- From 1990 to 2010, the number of females cared for at HTC's grew 346%



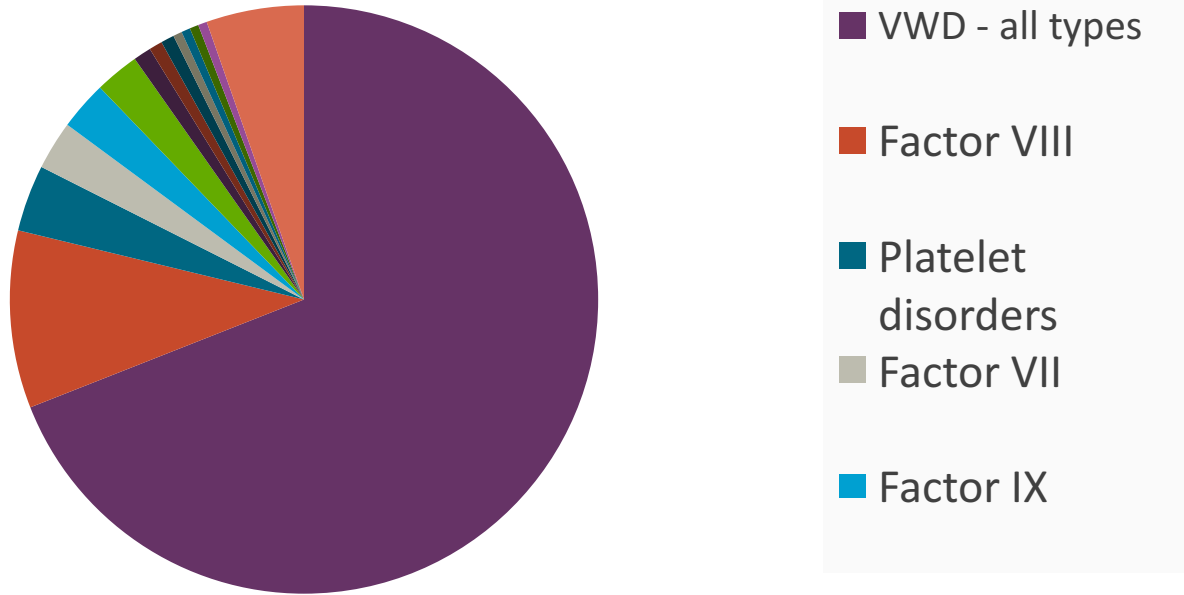
Baker JR, et al. Haemophilia. 2013

The Female UDC Project

- Added a module addressing female specific symptoms
- Aimed at improved surveillance of female patients
- Aimed at describing the burden of disease in female patients
- Data collected from September 2009 through December 2010
- 319 participants across 12 HTC's

Byams VR, et al. Haemophilia. 2011

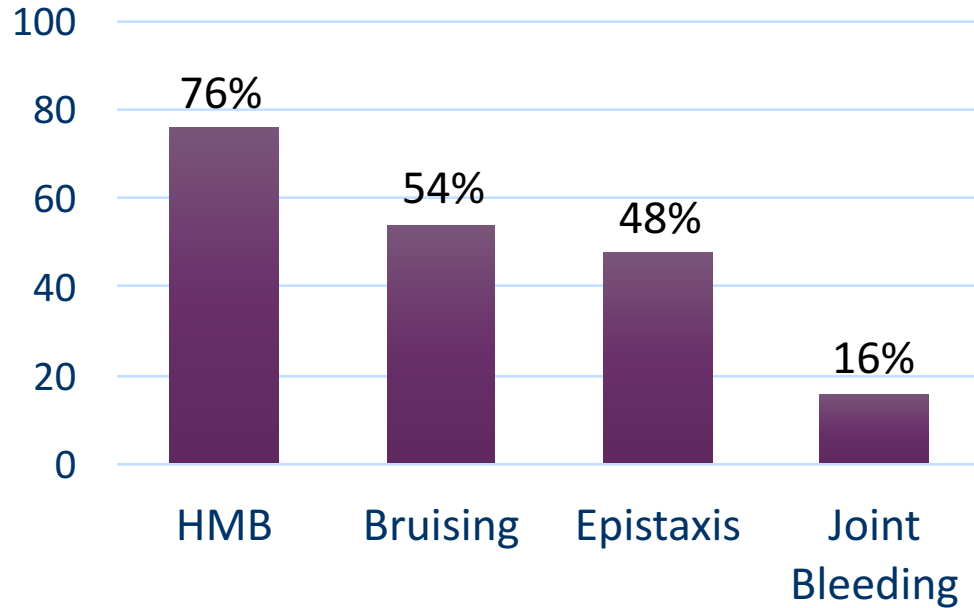
Diagnoses in the Female UDC Project



HHT – Hereditary Hemorrhagic Telangiectasia
CTD – Connective Tissue Disorders

The Female UDC Project

Of the 319 participants across 12 HTC's



- 53% of the 119 females who had been pregnant had pregnancy associated bleeding

Byams VR, et al. Haemophilia. 2011

ATHNdataset: the female population

Learning more about females with bleeding disorders

Study Design:

- Query of the ATHNdataset to identify the following information for female participants with bleeding disorders:
 - primary bleeding disorder diagnosis
 - demographic data
 - bleeding event information
 - treatment types
 - viral infection
 - mortality
- Descriptive statistics were employed

ATHNdataset: the female population

Study Design

- Definitions:

- Deficiency was defined as $<50\%$ for factors II, V, VII, VIII, IX, X, XI, XIII
- Deficiency was defined as < 200 mg/dL for fibrinogen
- Low VWF was defined as VWF:RCo or VWF:Ag of 30-50%

ATHNdataset: the female population

Study Design

- Definitions:
 - Rare Bleeding Disorders:
 - Factor II deficiency
 - Factor V deficiency
 - Combined factor V and VIII deficiency
 - Factor VII deficiency
 - Factor X deficiency
 - Factor XI deficiency
 - Factor XII deficiency
 - Factor XIII deficiency
 - Dysfibrinogenemia or afibrinogenemia
 - Hereditary Hemorrhagic Telangiectasia

Results: Demographics*

- 29,949 total patients enrolled in ATHNdataset
- 7,458 females (24.9% of the total ATHNdataset population)
- Median age of cohort was 21 years (IQR 15-37 years)

Ethnicity	N (%)
Non Hispanic	6480 (86.9)
Hispanic	892 (12.0)
Unknown	86 (1.2)

Race	N(%)
White	6360 (85.3)
African American	606 (8.1)
Asian	156 (2.1)
Mixed Race	71 (1.0)
Native Hawaiian or Pacific Islander	25 (0.3)
American Indian or Alaska Native	65 (0.9)
Unknown	175 (2.4)

*as of June 30, 2016

Results: Diagnosis*

Primary Diagnosis	N (%)
Von Willebrand Disease	4717 (63.3%)
Qualitative Platelet Disorder	1326 (17.8)
Hemophilia A	602 (8.1)
Rare Bleeding Disorder	597 (8.0)
Hemophilia B	216 (2.9)

*as of June 30, 2016

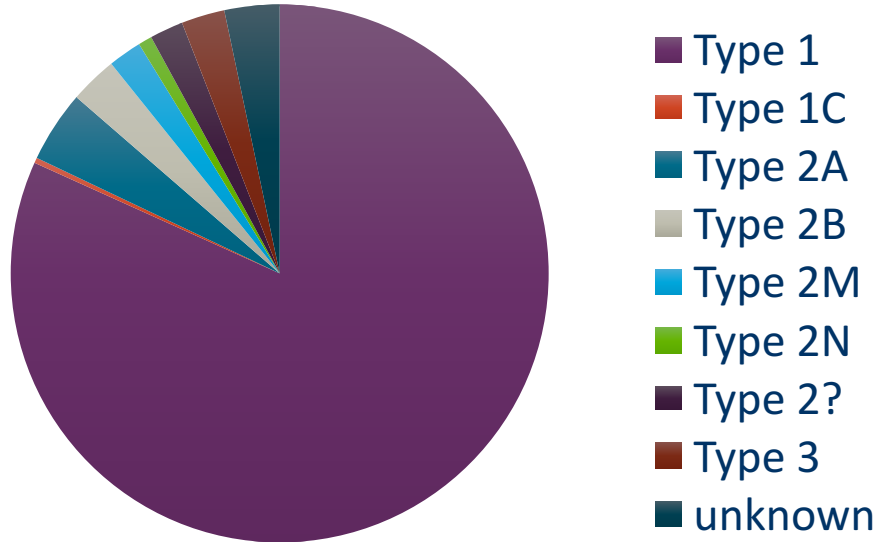
Results: Diagnoses

Age at diagnosis data:

- Median age at diagnosis of the 5606 participants with a known diagnosis age: 12 years
- Women with VWD or a Qualitative Platelet Disorder were more likely to be diagnosed before 18 years of age than all other diagnoses ($p < 0.001$)

Von Willebrand Disease

Total = 4717 patients



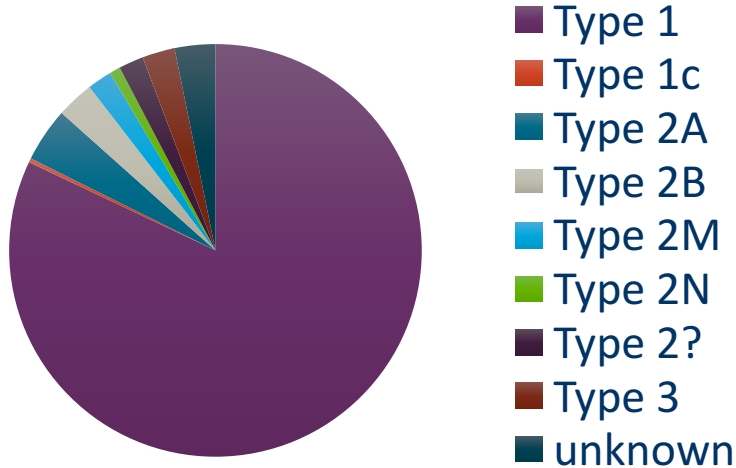
VWD diagnoses as reported

Diagnosis	N (% of all VWD diagnoses)
Type 1	3856 (81.7)
Type 1C	15 (0.3)
Type 2A	203 (4.3)
Type 2B	135 (2.9)
Type 2M	95 (2.0)
Type 2N	39 (0.8)
Type 2 unknown	95 (2.0)
Type 3	123 (2.6)
Unknown type	156 (3.3)

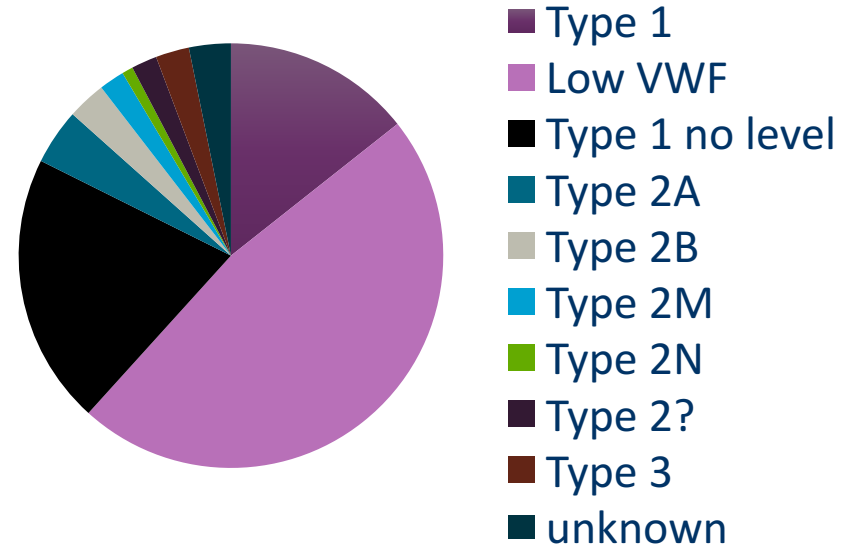
Von Willebrand Disease

- 2888 of the 3871 (74.6%) with type 1 VWD had a level reported
- 665 (23.0%) would meet NHLBI criteria for type 1 VWD diagnosis
- 2223 (77.0%) would be classified as having low VWF

Von Willebrand Disease



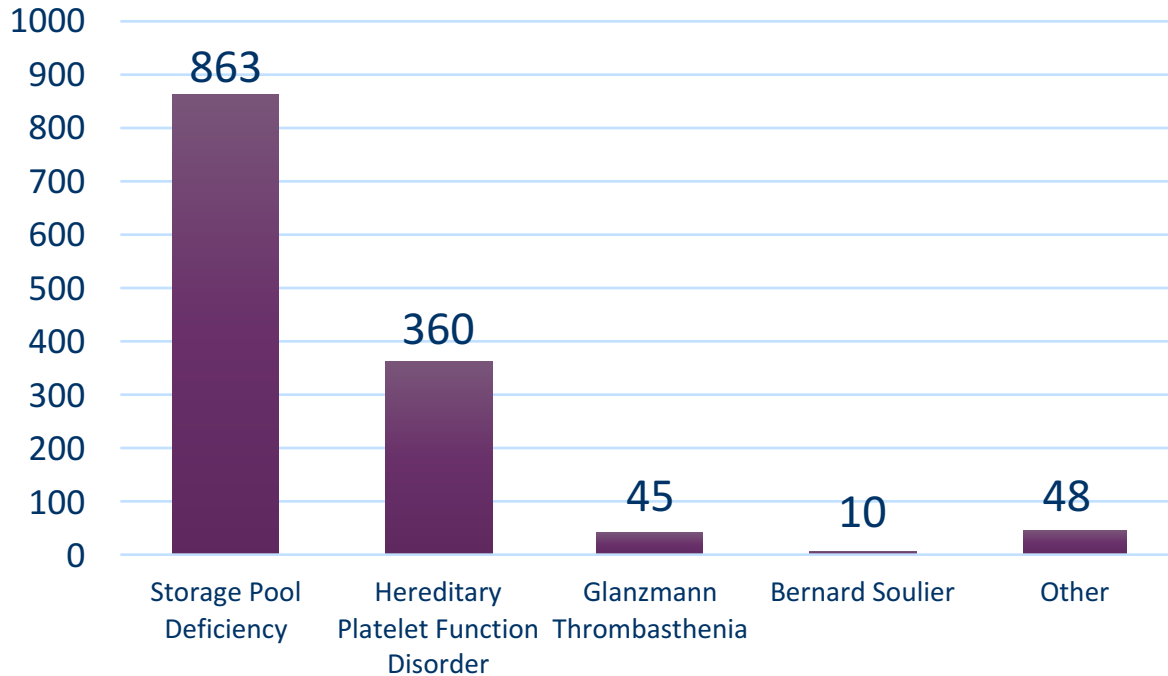
VWD diagnoses as reported



VWD diagnoses with levels

Qualitative Platelet Disorders

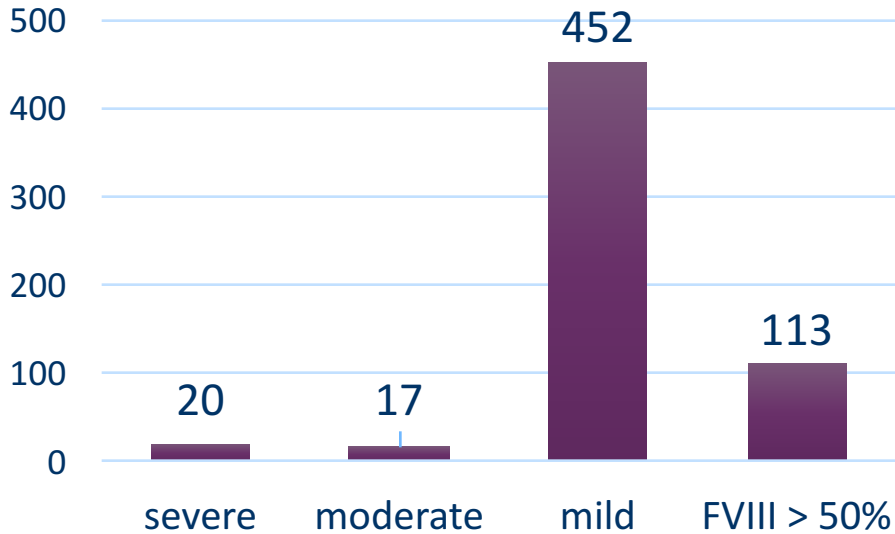
Total patients = 1326



- 48% of patients with QPD were children
- 77% of patients with a QPD were diagnosed before 18 years of age

Hemophilia A

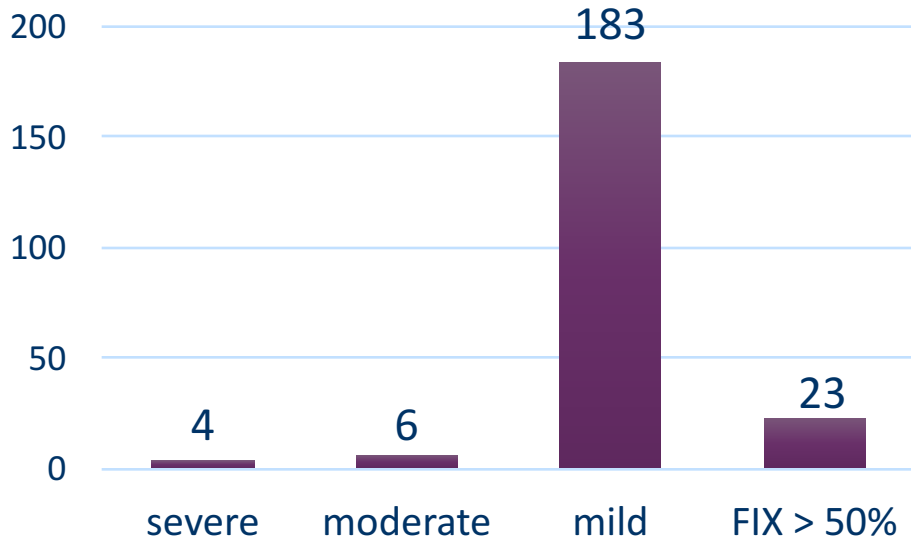
Total = 602 patients



Diagnosis	N (% of all Hemophilia A)
Severe	20 (3.2)
Moderate	17 (2.8)
Mild	452 (75.1)
Factor VIII > 50%	113 (18.8)

Hemophilia B

Total = 216 patients



Diagnosis	N (% of all Hemophilia B)
Severe	4 (1.9)
Moderate	6 (2.8)
Mild	183 (84.7)
Factor IX > 50%	23 (10.6)

Rare Disorders

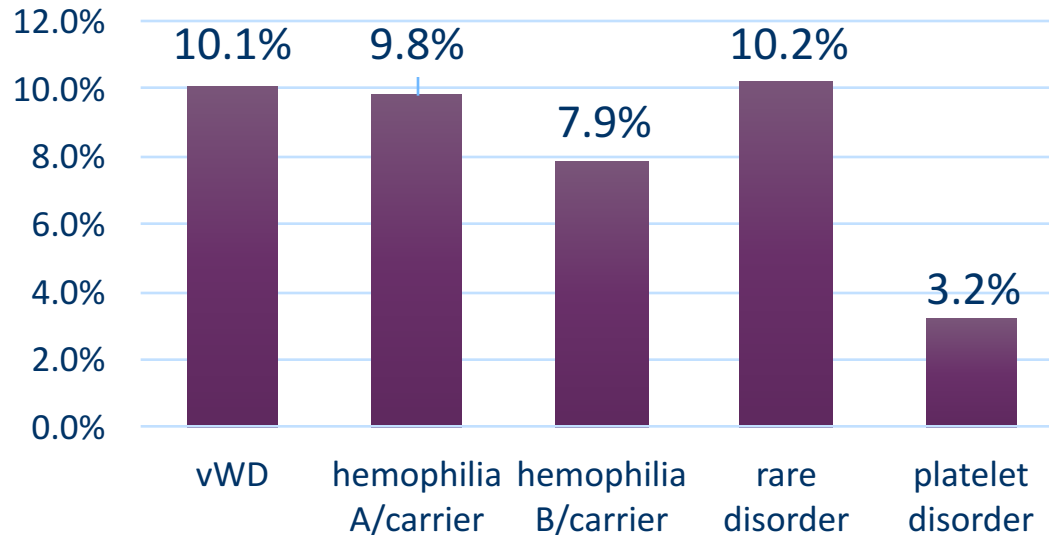
Total = 597 patients

The two most common diagnoses were factor VII and factor XI deficiencies:

- 237 patients had factor VII deficiency
- 140 patients had factor XI deficiency

Bleeding symptoms

- 658 (8.8%) of the cohort reported joint bleeding



Diagnosis	Number with joint bleeding
vWD	475
Hemophilia A	59
Hemophilia B	17
Rare disorder	61
Platelet disorder	46

Bleeding symptoms

- 826 (11.1%) reported reproductive tract bleeding
 - 812 reported heavy menstrual bleeding
 - 13 reported postpartum bleeding
 - 1 reported postmenopausal bleeding

Medical treatments used for reproductive bleeding

- Desmopressin – 411 patients
- Aminocaproic Acid – 346 patients
- Tranexamic Acid – 237 patients
- Oral Contraceptive – 180 patients
- Plasma – 149 patients (all with VWD)
- IUD – 14 patients
- Depo Provera – 14 patients

Conclusions

- The ATHN dataset is a robust database for capturing information about females with bleeding disorders
- VWD is the most common diagnosis in this cohort, but it is uncommon to meet NHLBI criteria for the diagnosis
- Reproductive tract bleeding rates are lower than expected
- Further work is needed to fully capture the impact of bleeding disorders as well as optimize diagnosis and treatment for females with bleeding disorders

This work was a collaborative effort:

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Thank you!