



Health Equity in the Context of New and Emerging Therapies for Hemophilia

Supported by an educational grant from Novo Nordisk, Inc.

CMEO **Snack 1**

Does Labeling the Level of Severity in Hemophilia Block Access to Optimized Care?



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Learning Objective

Provide equitable care for all patients with hemophilia, including those from underserved communities and those who do not meet formal severity thresholds for prophylaxis, yet experience significant bleeds.



Therapeutic Evolution

Factor Replacement

- Missing protein identified, purified, returned to PwH
- Viral inactivation
- Recombinant factor products
- Reduced volume
- Better storage/portability
- [FVIII, FIX concentrates]

Non-replacement, Rebalancing Therapies

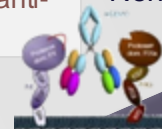
- Metabolic manipulation
- Small molecules; SC dose
- Use with or without inhibitors
- [FVIII mimetics, anti-TFPI, anti-APC, AT-siRNA]

AAV vectors

2017–2020s
Nonfactor treatment

2010s–2020s
Gene treatment

1990s
Recombinant FVIII/FIX



2014
EHL factors

Gene Therapy

- Provides functional gene or edits abnormal gene
- Potential long-term cure or remission
- [FVIII and FIX products FDA approved]

Extended Half-life (EHL)

- Less frequent infusions
- Improved adherence
- Higher trough activity
- Better bleed protection
- [EHL rFVIII, EHL rFIX]

1968

Commercially available FVIII

1985

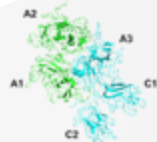
Viral inactivation

1964

Cryoprecipitate

1950s–1960s

Fresh frozen plasma



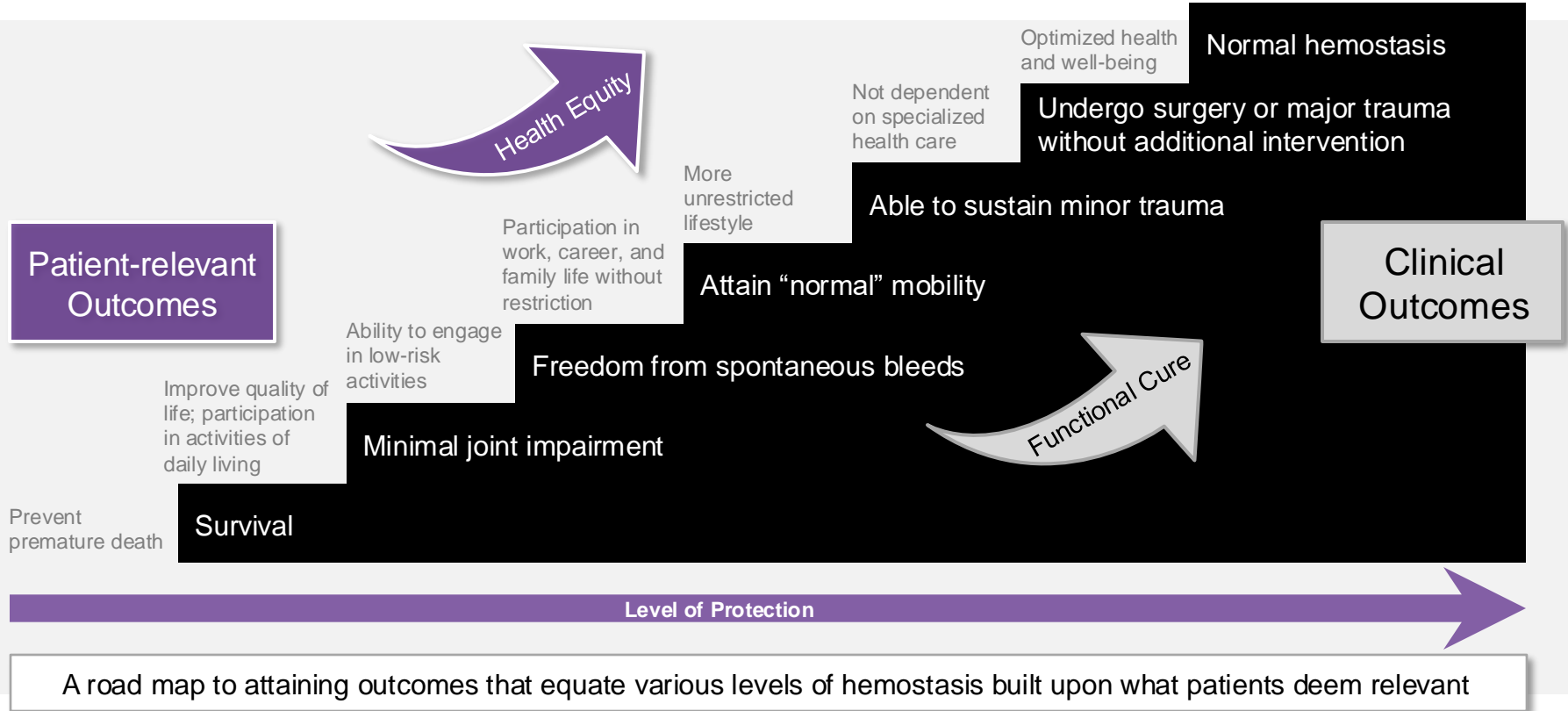
1900–1940s

Whole blood

APC, activated protein C; AT, antithrombin; FIX, factor IX; FVIII, factor VIII; PwH, person with hemophilia; r, recombinant; RNA, ribonucleic acid; SC, subcutaneous; si, small interfering; TFPI, tissue factor pathway inhibitor.

Ozelo MC, Yamaguti-Hayakawa GG. *Res Pract Thromb Haemost.* 2022;6:e12695.

Achieving the Unimaginable: Health Equity



Patient Case: Lucas

- Lucas, 4-year-old Black male with mild (5%) hemophilia A

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Severity	Clotting factor level	Bleeding episodes
Severe	<1 IU/dL (<0.01 IU/mL) or <1% of normal	Spontaneous bleeding into joints or muscles, predominantly in the absence of identifiable hemostatic challenge
Moderate	1-5 IU/dL (0.01-0.05 IU/mL) or 1-5% of normal	Occasional spontaneous bleeding; prolonged bleeding with minor trauma or surgery
Mild	5-40 IU/dL (0.05-0.40 IU/mL) or 5-<40% of normal	Severe bleeding with major trauma or surgery; rare spontaneous bleeding

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- Presents with a significant ankle bleed accompanied by severe pain that requires pain medication and hospitalization for 2 days

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- Presents with a significant ankle bleed accompanied by severe pain that requires pain medication and hospitalization for 2 days
- This is Lucas' first bleed
- Parents did not notice any specific trauma – only Lucas' usual “safe” playtime activities

What were your thoughts when managing this patient, particularly with regard to on-demand therapy vs prophylaxis?



Can you describe the “vicious cycle” of how one bleeding episode can portend additional bleeds?

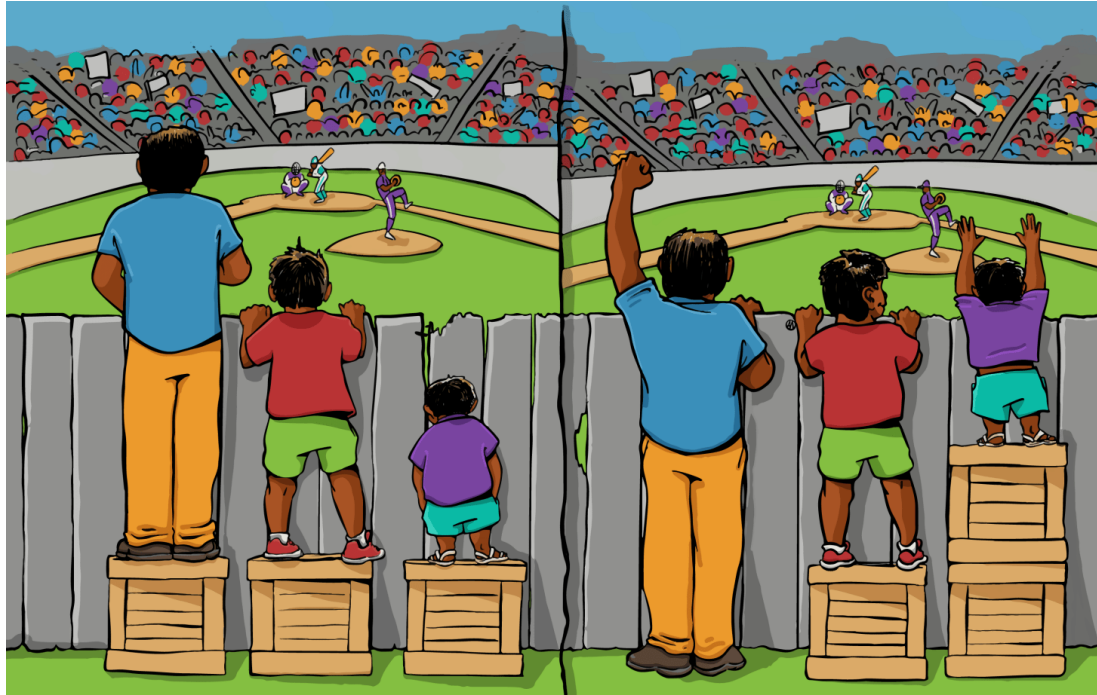


Health Equity: Definition

“The attainment of the highest level of health for all people.”



Equality vs Equity



EQUALITY

EQUITY

Image courtesy of "Interaction Institute for Social Change" | Artist: Angus Maguire. www.interactioninstitute.org and madewithangus.com. Accessed June 14, 2024..

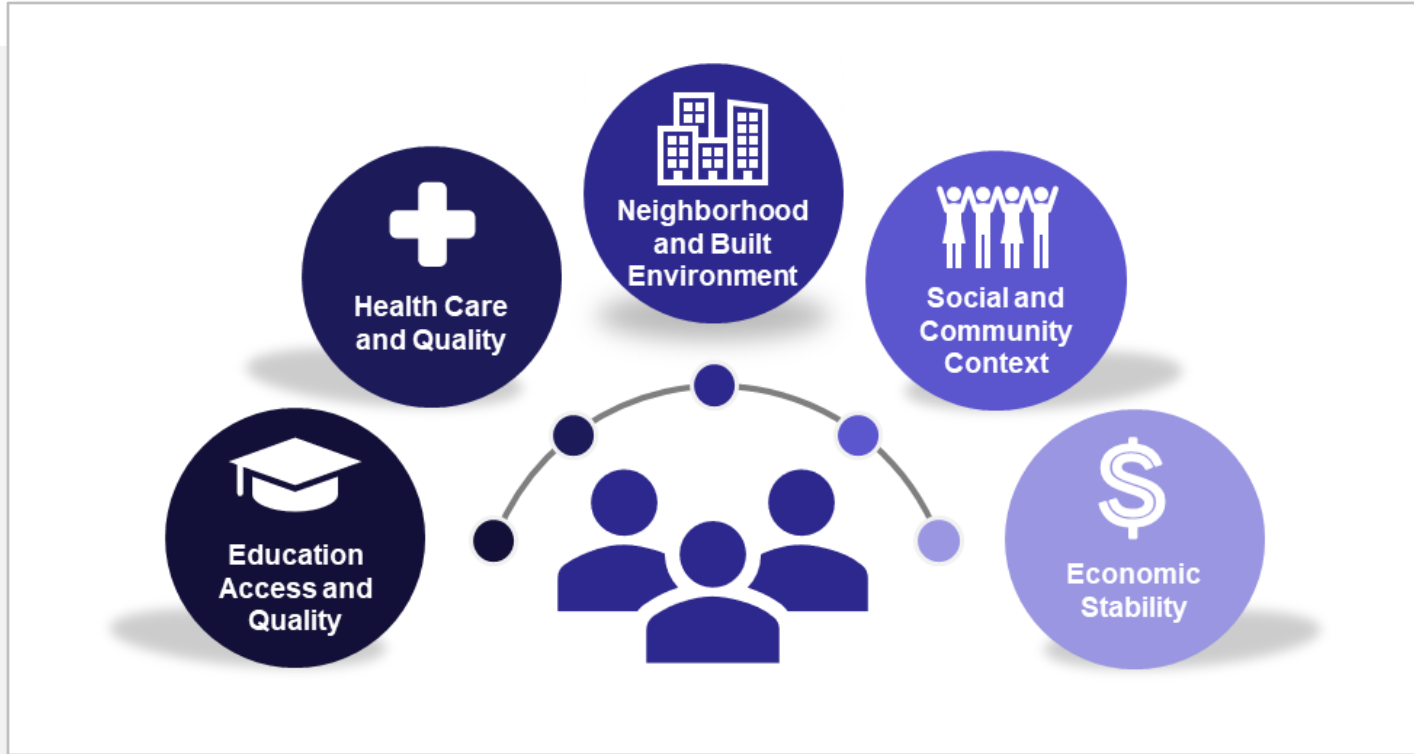
Health Disparities

“A particular type of health difference that is closely linked with social, economic, and/or environmental disadvantage.”

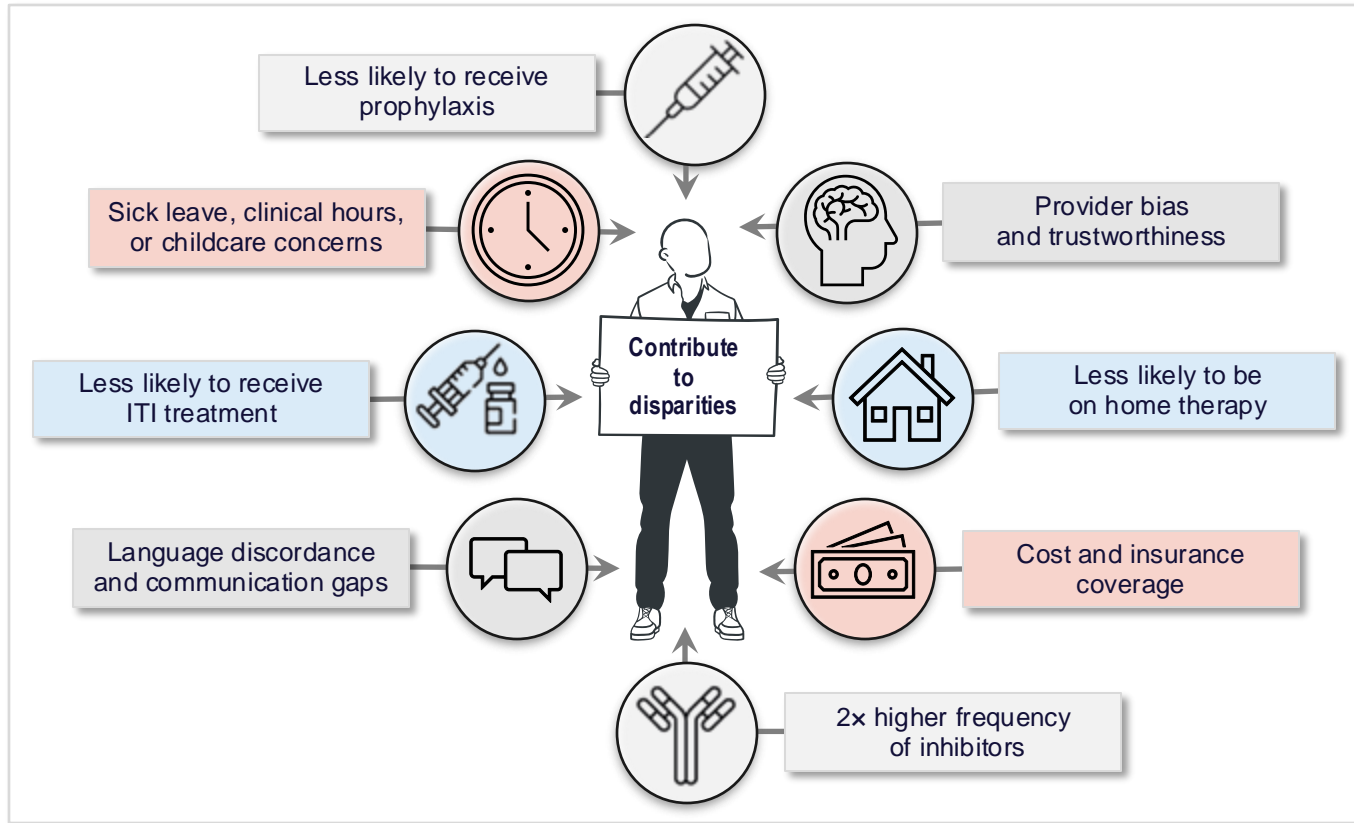
Adversely affect groups of people who have systematically experienced greater obstacles to health based on their:

- Racial or ethnic group
- Religion
- Socioeconomic status
- Gender
- Age
- Mental health
- Cognitive, sensory or physical disability
- Sexual orientation or gender identity
- Geographic location
- Other characteristics historically linked to discrimination or exclusion

Social Determinants of Health

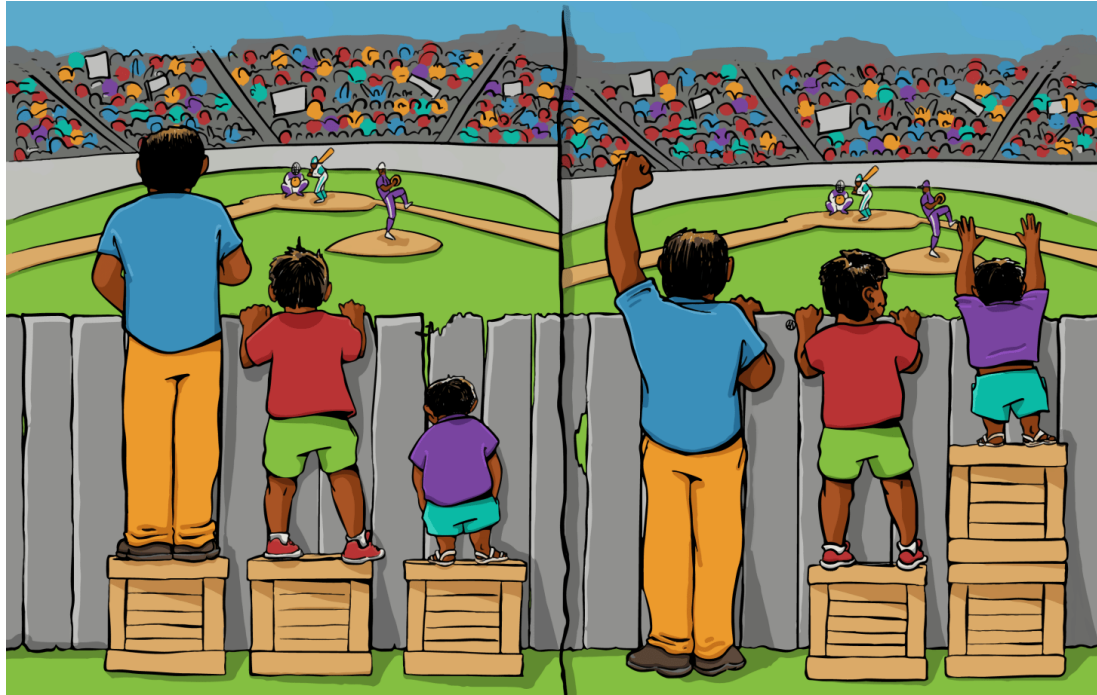


Contributors to Racial and Ethnic Disparities in Hemophilia Care and Outcomes



Do you make treatment decisions based on health literacy or do you spend more time educating patients, so they are fully prepared to start a certain therapy?

Equality vs Equity



EQUALITY

EQUITY

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What is Shared Decision Making?

A process wherein:

A patient shares with the provider all their aspirations, relevant values, preferences, and goals.

A health care provider shares with a patient all relevant information and best scientific evidence on the pros and cons of all potential treatment options.

With this mutual understanding, the **patient and provider decide** the best course of action.

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SMART Goals

Specific, Measurable, Attainable, Relevant, Timely

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- Stay current with new and emerging therapeutic options for hemophilia
- Take steps to ensure equitable care is provided to all patients
 - Assess and address SDoH
 - Engage in SDM
 - Work with patients/families to access optimized care

To Receive Credit

To receive CME/CE credit for this activity, participants must complete the post-test and evaluation online.

Participants will be able to download and print their certificate immediately upon completion.



Coming soon...

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Racial/Ethnic Disparities in the
Context of New/Emerging
Treatments for Hemophilia

