



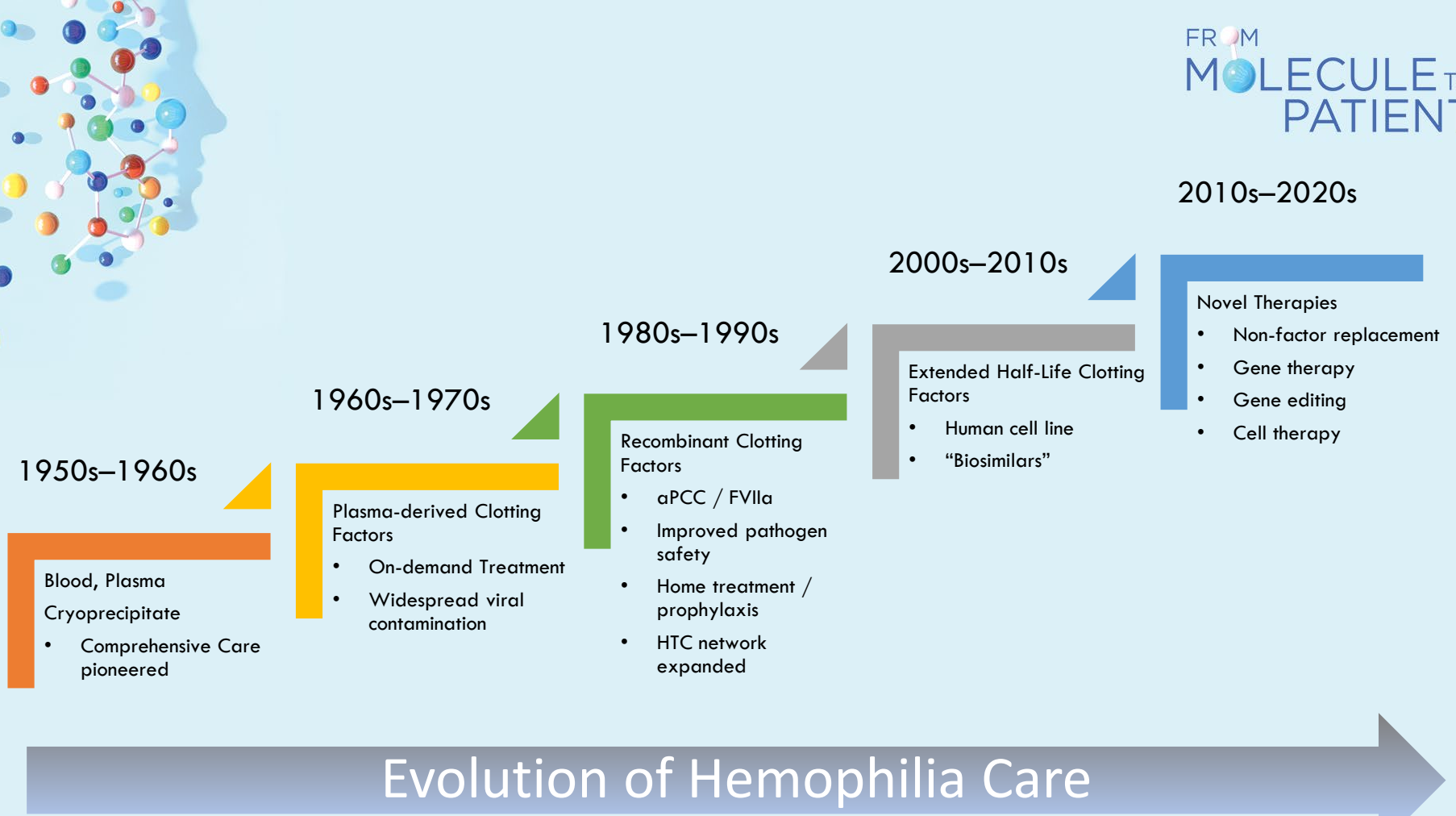
Engaging Patients Throughout the Life Cycle of Drug Development

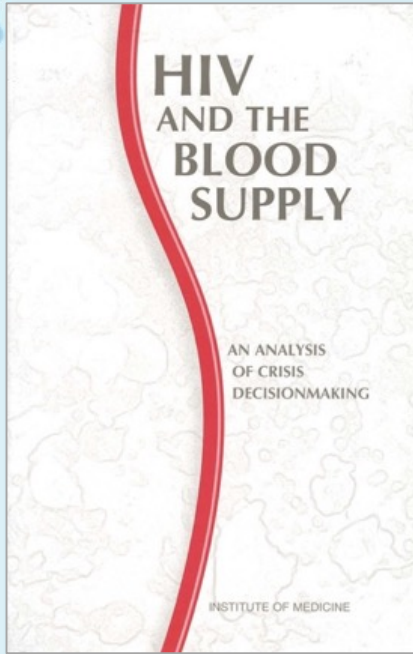
Mark W Skinner, JD
ASCPT 2019
16 March 2019
Washington, DC

Conflict Disclosures

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Advisory Committee	US HHS ACBTSA, Bayer, Blue Cross Blue Shield MAP, NHF MASAC, Roche/Genentech, Pfizer (DMC), Spark (DSMB)
Consultant	NHF







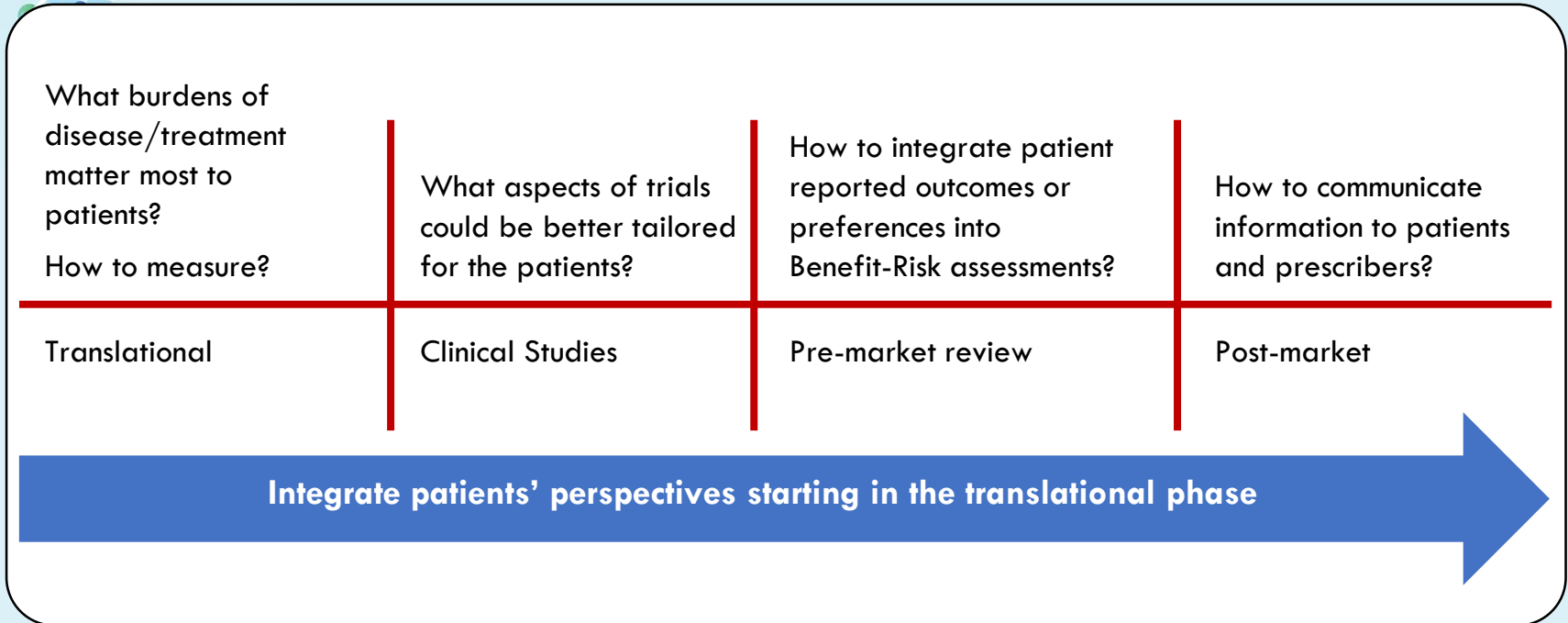
*Blood safety is a **shared responsibility** of many diverse organizations. They include ... manufactures, **groups like the NHF**, and others.*



Patients have a unique perspective and will consider issues differently than regulators, manufacturers, scientists, clinicians and payers.



Patient-Focused Drug Development



The NEW ENGLAND JOURNAL of MEDICINE

Perspective

Standardizing Patient Outcomes Measurement

Michael E. Porter, Ph.D., M.B.A., Stefan Larsson, M.D., Ph.D., and Thomas H. Lee, M.D.

The arc of history is increasingly clear: health care is shifting focus from the volume of services delivered for patients, as the outcome to the costs. It has been slow at first, but now it is clear that matter to survival, many conditions have different outcomes which are measured differently.

Providers to embrace accountability for results.

If we're to unlock the potential of health care, we need to

example, only 139 (7%) are actual outcomes and only 32 (<2%) are patient-reported outcomes (see bar chart).

Perspective

DECEMBER 23, 2010

What Is Value in Health Care?

Michael E. Porter, Ph.D.

In any field, improving performance and accountability depends on having a shared goal that unites the interests and activities of all stakeholders. In health care, however, stakeholders have

myriad, often conflicting goals. Value — neither an abstract ideal nor a code word for cost

value is a central challenge. Nor is value measured by the process of care used; process measurement and improvement are important tactics but are no substitutes for measuring outcomes and costs.

Since value is defined as out

- What matters to patients are outcomes that encompass the whole cycle of care
- Historically, outcomes measurement has focused on clinical status and left out functional status

Value in Healthcare = Value Created for Patients

Patient Reported **Relevant** Outcomes

Traditional Clinical Outcomes	Patient-Centric Outcomes
Lifespan (survival)	Educational pursuit
Factor levels (peaks & troughs)	Work / Career opportunities
Bleeding frequency (annualized bleed rate, target joints)	Family / Social life engagement
Joint outcomes (structure, range of motion)	Activity / Sports goals
Function and mobility	Decreased burden of illness

It is clear that although there have been great advances ..., more needs to be done not only to develop new therapies ..., but to address broader economic, social, and educational barriers that still remain.

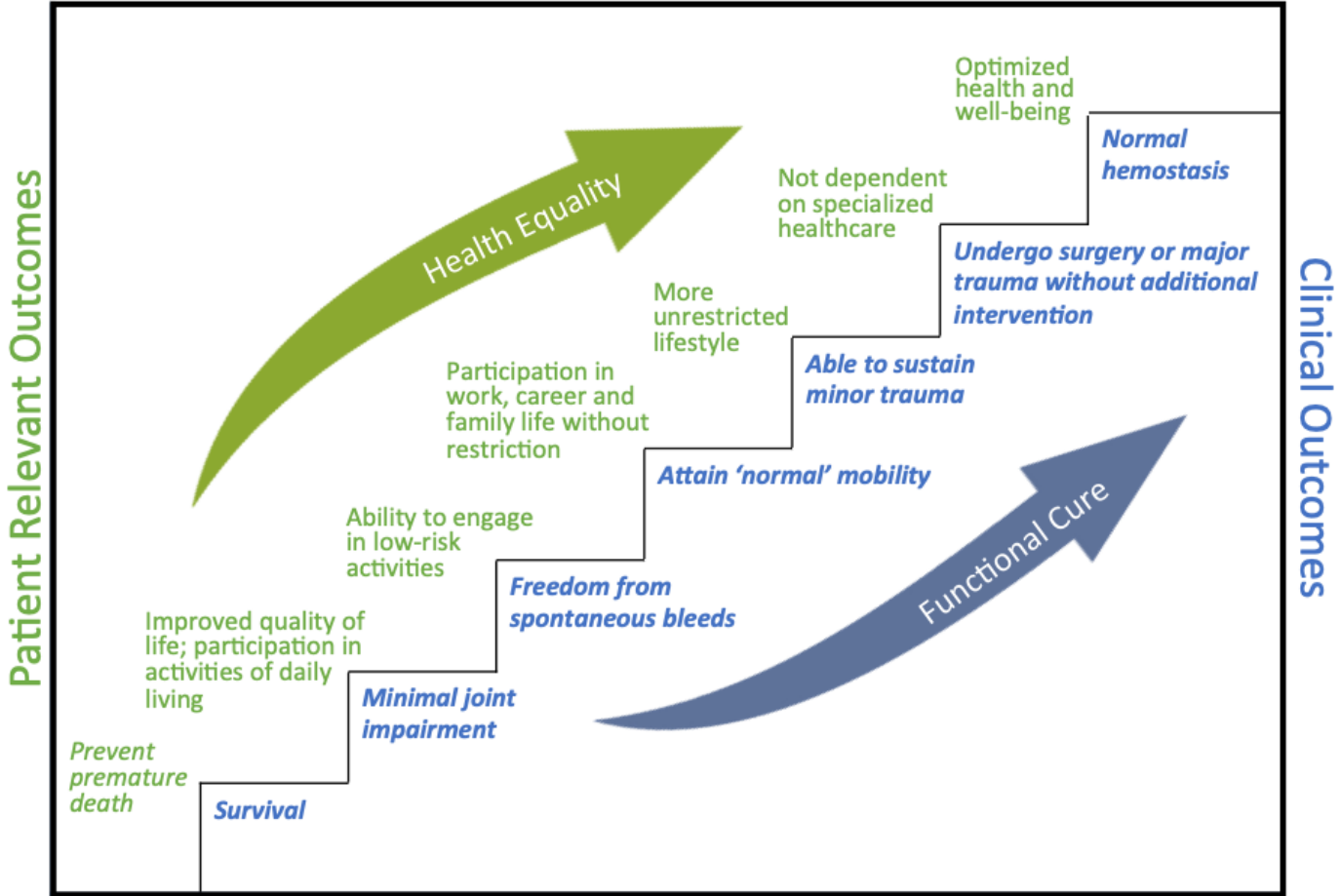
FDA Voice of the Patient
Report Conclusion
May 2016

Comparing Outcomes Patients Deem Relevant

- Pain - chronic/acute, interference, occurrence
- Independence - limitations, impact on activities of daily living
- Education - attainment, attendance
- Employment - duration, underemployment, attendance
- Family life - marriage, children
- Mobility - assistance required, impairment
- Current health status (EQ-5D-5L – VAS)



Achieving the Unimaginable: Health Equity



Core Outcomes

- Frequency of bleeds
- Factor activity level
- Duration of expression
- Chronic pain
- Mental health status
- Utilization of healthcare system (direct costs)

Additional Outcomes

- Duration/frequency/type of physical activity/sport/play
- Physical health/general health perception

Adverse Events

- Short-Term, Long-Term, Mortality

Collect and report
well specified
outcomes within
clinical trials

Market Authorization

Increase predictability
and consistency of payer
/ HTA appraisal when
making coverage
decisions

Market Access

Shared decision-making
using outcomes meaningful
to the quality of life and
functioning of patients

On-Market Use

Consistent collection and reporting of relevant and well-specified outcomes

Enhance the Patient Voice Across the Life Cycle

Patients as subjects
(passive recipient)

Patients as partners
(actively involved)

Patient engagement will drive the development of innovative medicines that deliver more relevant and impactful patient outcomes and make medicine development faster, more efficient, and more productive.