

---

# PERSONALIZED MEDICINE AND VALUE ASSESSMENT FRAMEWORKS

Context, Considerations, and Next Steps



*Personalized medicine, also called precision or individualized medicine, is an evolving field in which physicians use diagnostic tests to identify specific biological markers, often genetic, that help determine which medical treatments and procedures will work best for each patient.*

## In this report we...

Describe the intersection of personalized medicine and VAFs

Provide an overview of U.S.-centered VAFs

Identify areas of consideration related to personalized medicine that need to be accounted for in VAF methodology

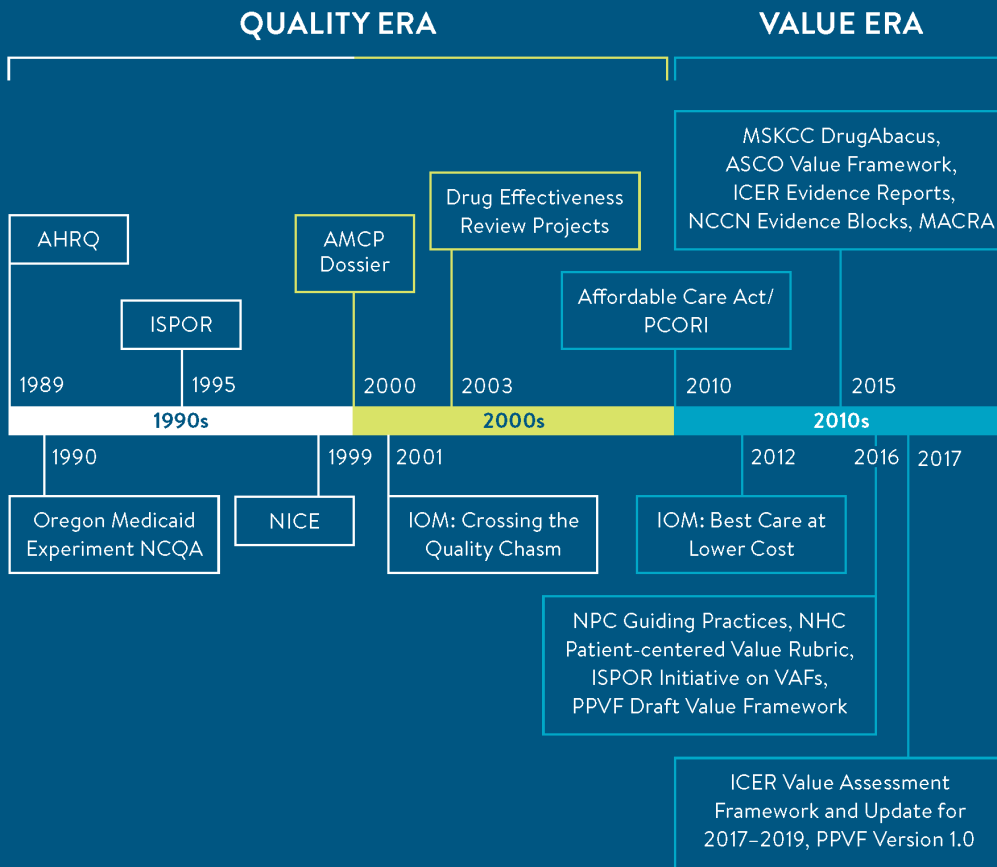
Provide a synopsis of how VAFs may incorporate these considerations.

Offer recommendations for refining VAF to better align with the value of personalized treatments to both patients and the health care system.

# Context of personalized medicine and value assessment

*Challenges in the uptake and integration of personalized medicine*

FIGURE 1. EVOLUTION OF VALUE-BASED HEALTH CARE IN THE UNITED STATES



- 2006 – 2016 roughly 22-fold increase in personalized medicines, diagnostics, and treatments available

# Context of personalized medicine and value assessment

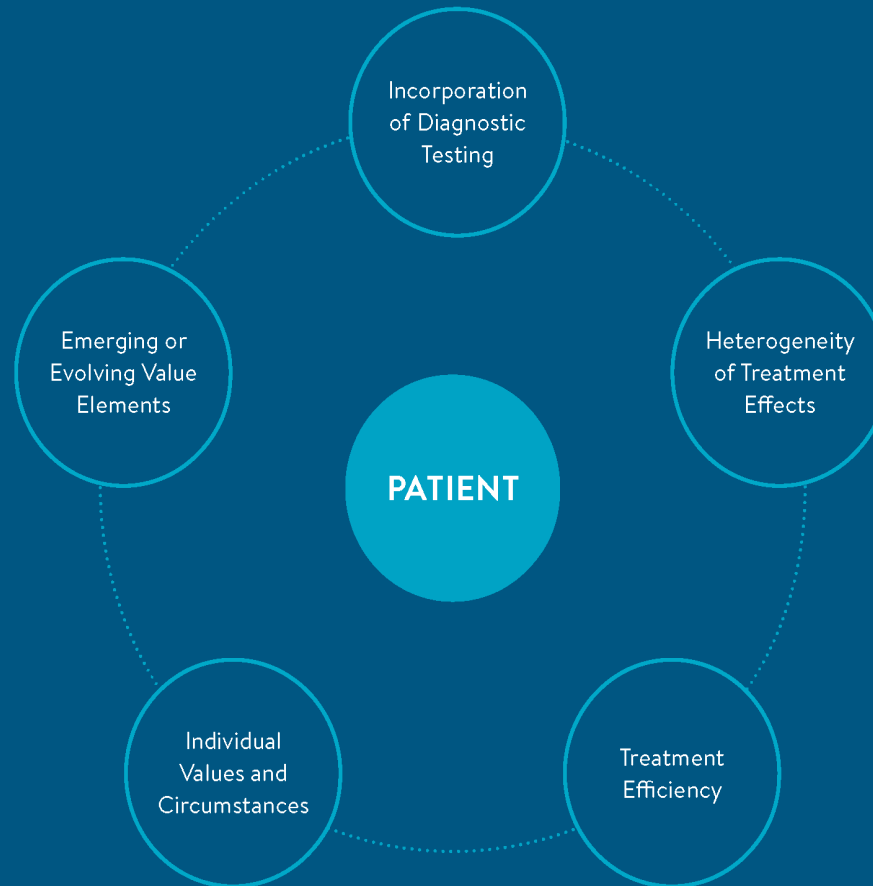
*Challenges in the uptake and integration of personalized medicine*

**TABLE 1. CHALLENGES TO THE UPTAKE AND INTEGRATION OF PERSONALIZED MEDICINE IN CLINICAL PRACTICE INCLUDE BARRIERS RELATED TO VALUE DEMONSTRATION**

<b>Knowledge and Empowerment</b>	Lack of education and awareness among patients and health care professionals
	Lack of patient empowerment
<b>Value Demonstration</b>	Lack of value recognition (both clinical and economic) and evidence requirements for coverage
	Uncertain evidence requirements for coverage/ reimbursement and other access hurdles
	Access and reimbursement hurdles
<b>Infrastructure</b>	Lack of effective health care delivery infrastructure and information management systems

*VAFs have the potential to encourage the use of personalized medicine, but that can only happen if frameworks incorporate the elements of personalized medicine that demonstrate its value*

FIGURE 2. NECESSARY PERSONALIZED MEDICINE CONSIDERATIONS FOR VAFS



## 5 Strategic Recommendations to Unite Value Assessment and Personalized Medicine



Diagnostic testing must be considered an integral part of the assessment of the value of treatment options where efficacy and/or safety information can be obtained.

A formal mechanism for consideration of heterogeneity of treatment response needs to be appropriately balanced with population-based considerations.

Methods for assessing value must overtly consider emerging or evolving elements over time to elucidate the benefits of potentially useful treatments on an individual patient level.

- Frameworks must be explicit in the patient population being evaluated.
- Frameworks must frequently update or re-evaluate the treatment landscape in order to capture patient groups that may attain more value from treatments than others through factors associated with the practice of personalized medicine.



## 5 Strategic Recommendations to Unite Value Assessment and Personalized Medicine

*Continued*

Education and awareness of appropriate application and use of value assessment frameworks in personalized medicine, including approaches that provide greater transparency and capacity for disaggregation of results that are being applied at the population level, must be increased among payers to reduce the risk of inappropriate restrictions on reimbursement and access to medicines that lead to limited patient access to individualized care.

The perspectives of all personalized medicine community stakeholders, especially patients, must be considered in the development of VAFs and the assessment of therapies.

Thank you

*Kristen Migliaccio-Walle*

Director, GHEOR

Xcenda, LLC