Early MA Assessment for personalized medicine (PM):

a framework to assess the challenges on the diagnostic side

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healthcare consultants

Outline

1. **How is this topic relevant to you**
2. The case for an early Market Access assessment
3. How we put a Framework to work: Case studies
4. Key takeaways

How is this presentation relevant to you?

Do you have / will you soon have a Personalized Medicine solution in the scope of your responsibility?

Even if you do not have a PM solution today, there are chances you will have one in the near future (in pipeline or in market)

The PM market is growing: more drugs are developed together with biomarkers and more make it to official national lists

166 drugs in the PGx list by the FDA

+18% since 2015 (new launches and labels updates)

43 drugs in the PM list by the VdA

+13% since 2015

Biopharmaceutical firms expect PM in development to increase 69% over the next 5 years[1]

Mentioned in 1 or more section of the Label: Boxed Warning, Indications and Usage, Dosage and Administration, Adverse Reactions, Clinical Pharmacology, Clinical Studies, Warnings and precautions, Drug Interactions, Clinical Pharmacology, Use in Specific Populations

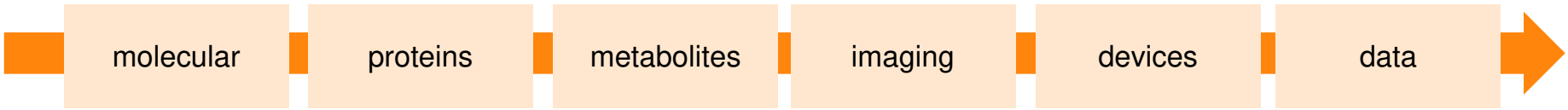
PGx: Pharmacogenetics, including combination treatments
http://www.fda.gov/Drugs/ScienceResearch/ResearchAreas/Pharmacogenetics/ucm083378.htm
https://www.vfa.de/de/arzneimittel-forschung/datenbanken-zu-arzneimitteln/individualisierte-medizin.html
[1]Tufts Center for the study of Drug Development, "Personalized Medicine gains traction but still faces multiple challenges", Impact Report, May/June 2015, Vol. 17, N. 3

…even more so as the definition of PM is expanding beyond genomics approaches and efficacy/toxicity applications

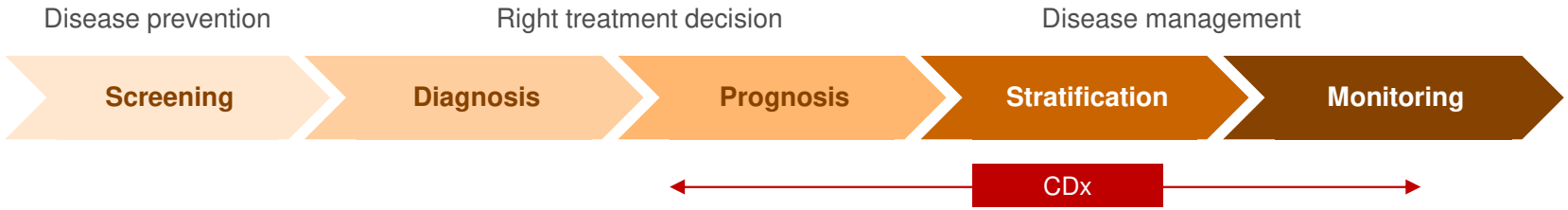
Personalized medicine is more than molecularly targeted treatments. It increasingly refers to a broader notion of ***"providing the right treatment to the right patient, at the right dose at the right time"*** *(European Union) using one or more of a multitude of* ***tools and technologies***

"A form of medicine that uses information about a person's ***genes, proteins, and environment*** *to* ***prevent, diagnose, and treat*** *disease." National Cancer Institute, NIH*

Multiple approaches



Multiple application



CDx = Companion Diagnostics, Dx = Diagnostics

A “Beyond the pill” solution could also be considered PM and be the object of an assessment to test access & reimbursement

A “Beyond the pill” solution could apply as Personalized Medicine and therefore be reimbursed/funded if:

- It proves to have benefit in combination with the drug and vs. SoC during Ph III studies
- It proves to have benefit in combination with the drug and vs. SoC in RWE studies
- It proves to work for the condition in scope in independent studies

By Beyond the pill, we mean any tool and technology that help providing a personal solution to the patient that will enhance efficacy and therefore outcome

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PM products are characterized by a high level of unknown, long timelines and a different appreciation of value

On top of what is generally required for drugs, Dx, devices, etc. have an additional layer of complexity when it comes to the assessment, funding, evidence requirements

Ex: MA challenges faced by CDx / Dx

Assessment process is different and not aligned with that of Rx

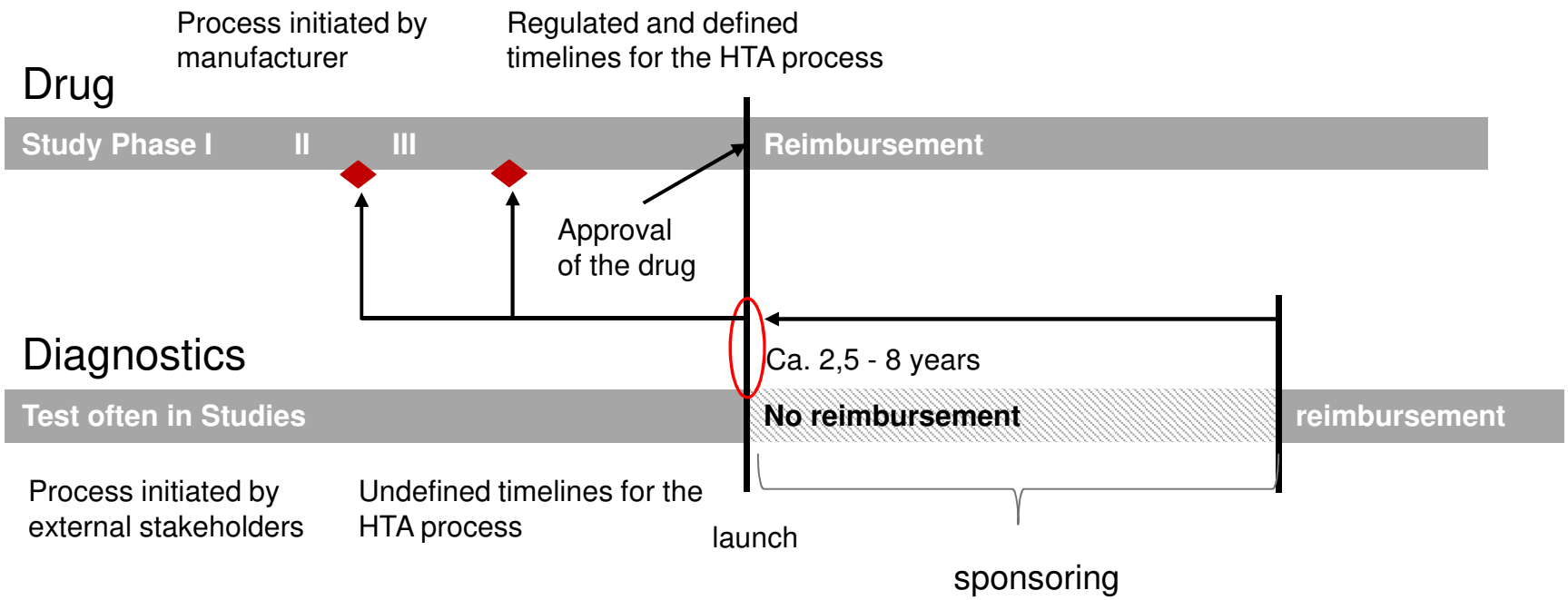
Assessment process is not driven by manufacturer and has no deadlines (Black box)

Evidence requirements are increasing* yet not transparent and uniform

Reimb. follows a cost based and not benefit based logic and is often inadequate

*prove that the new treatment strategy, including the new test, provides a better medical benefit and that there is an appropriate cost-effective relation

These challenges can result into a hurdle for the successful launch and uptake of the drug



It took 4 years in Germany to reimburse HER2

Dx-related MA activities should be initiated as early as possible: before Phase III, with another touchpoint before the initiation of the Rx HTA process to reduce the funding gap and optimize access and revenues

An early assessment allows to uncovers challenges & opportunities and provides input into critical decisions and activities

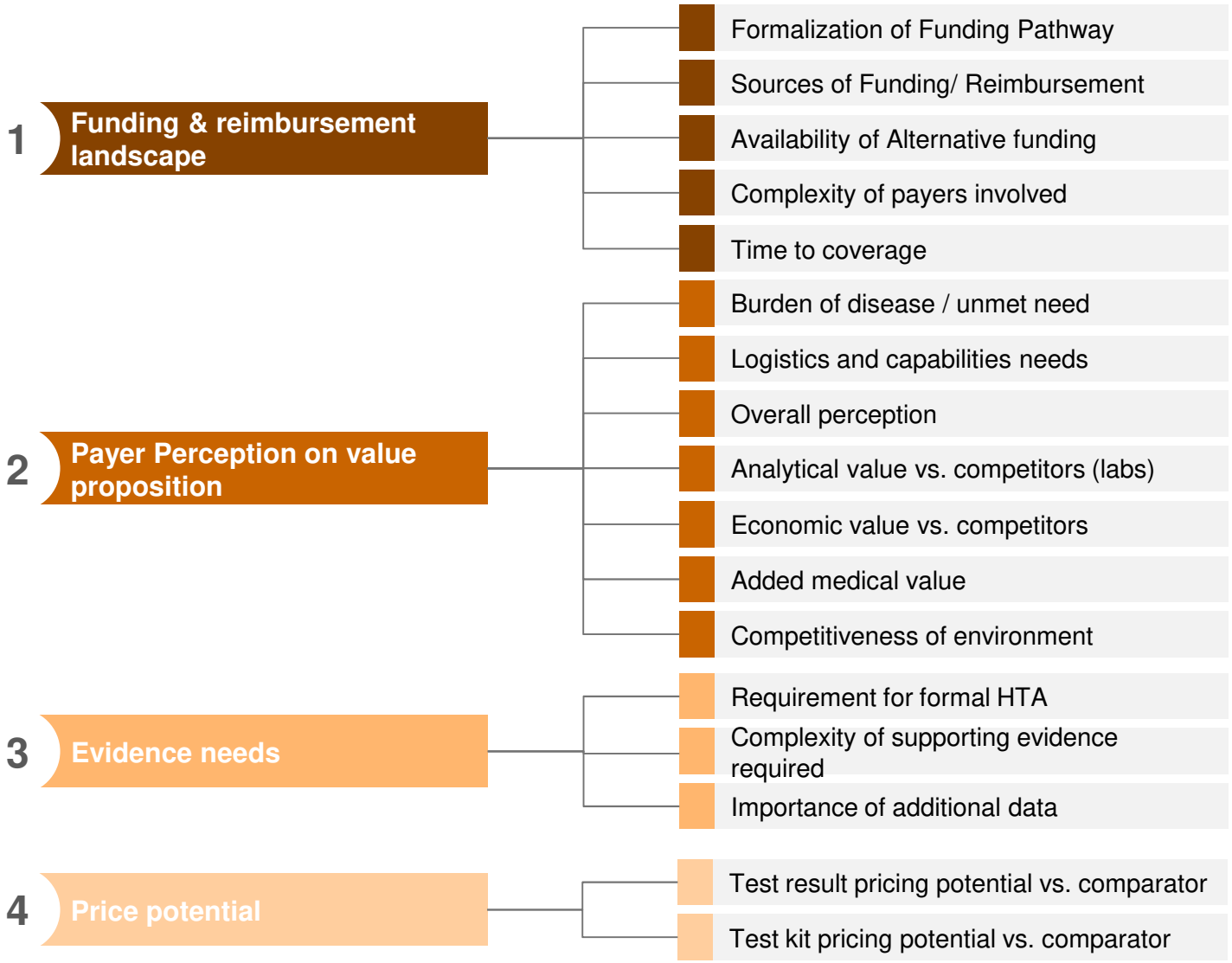
Overview of the framework used to assess the diagnostic part of a PM solution

Funding & Reimbursement landscape	Payer perception of value proposition	Evidence needs	Price potential
• *How is the **funding / reimbursement** landscape going to look like?*	• *Which **Value Proposition** is going to support reimbursement?*	• *What **evidence** supports the value proposition/will generate positive appraisal?*	• *What **price** is reasonable for payers and the system?*

Provides input into

decisions	Trial design, label and other evidence	Value Proposition	Reimbursement bridging strategies	Logistics/ accessibility solutions
activities	Payer engagement activities	Partner alignment activities	Stakeholder management activities	Policy shaping activities

The early assessment investigates 17 parameters with different weighting depending on the scenarios the product is into



Possible scenarios:

- Reimbursement: Yes / No
- Type of product: New / known product but not established / established product with different technologies available

Each parameter is linked to one or multiple questions that are rated according to defined variables and scales

RATING METHODOLOGY

Parameter	Question/s	Variable rated	Scoring
TIME TO COVERAGE	What will be the average time to coverage for such a test in your country?	≤1 >1≤3 >3	≤1 = 5 >1≤3 = 3 >3 =1
REQUIREMENT FOR FORMAL HTA	For the reimbursement of a new test, would a formal HTA assessment be: • mandatory • not mandatory but recommended • not mandatory and not recommended	• mandatory • not mandatory but recommended • not mandatory and not recommended	• not mandatory and not recommended = 5 • not mandatory but recommended = 3 • mandatory = 2

Questions, variables and scoring system were validated and adjusted in multiple projects together with the commissioning client

The process is designed to be pragmatic as usually this analysis comes on top of the assessment for the drug

Month 1	Month 2	Month 3

Landscape & Product analysis → **Stakeholder input collection** → **Scoring & Conclusions**

Country	Payers*	Lab head	KOL	Experts**	TOT
A	1-2	1-2	1-2	1	4-7
B	…	…	…	…	…
C	…	…	…	…	…

*Payers can be national, regional or local depending on the system and the product in scope

**Experts can be for example in coding or policy, regulatory.

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Case 1. A PM solution for a severe respiratory condition combining a drug with a novel immuno-based diagnostic test

1. Delayed and insufficient funding

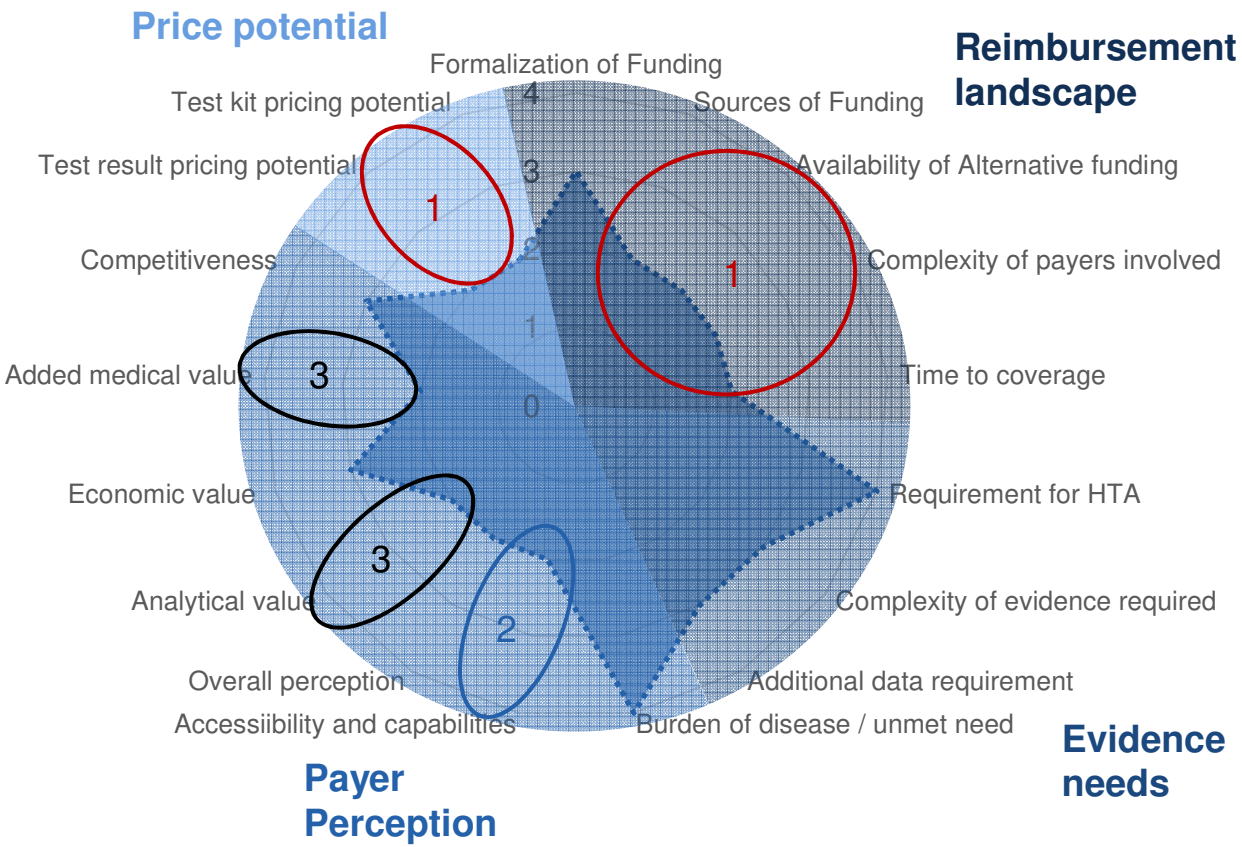
→ Temporary funding / sponsorship solutions / renegotiation with Dx partner (depending on countries)

2. Test runs on specific machines with low installed base

→ Need to create lab networks and system of referral to ensure accessibility

3. Uncertainty around test thresholds and combination of results with other tests

→ Decision to launch the product 2 years earlier to shape image and gain experience



Note: The rating is provided from the company perspective 1 = least favorable scenario and 5 = most favorable scenario

Case 2. A PM solution for a neurological condition combining a drug with a non genetic IVD test already available in market

1. Evidence development plan considered incomplete

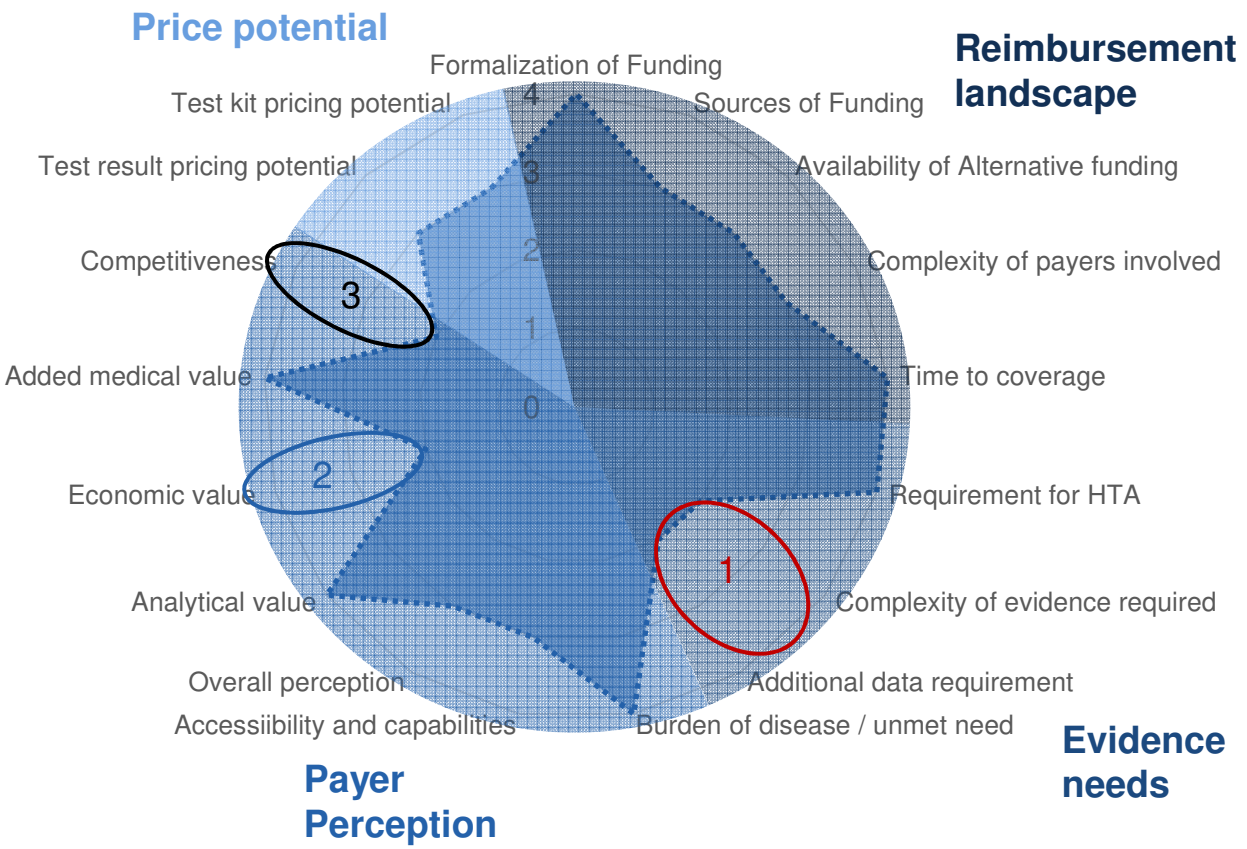
→ Generate evidence also vs. manual test

2. Test not broadly reimb. and payers are afraid of the explosion in use due to the high n. of patients

→ Capping agreements with payers national payers and cost benefit models with hospitals

3. Competitor solutions are perceived as more performing yet they generate problems of capacity and costs

→ BIM / CE models



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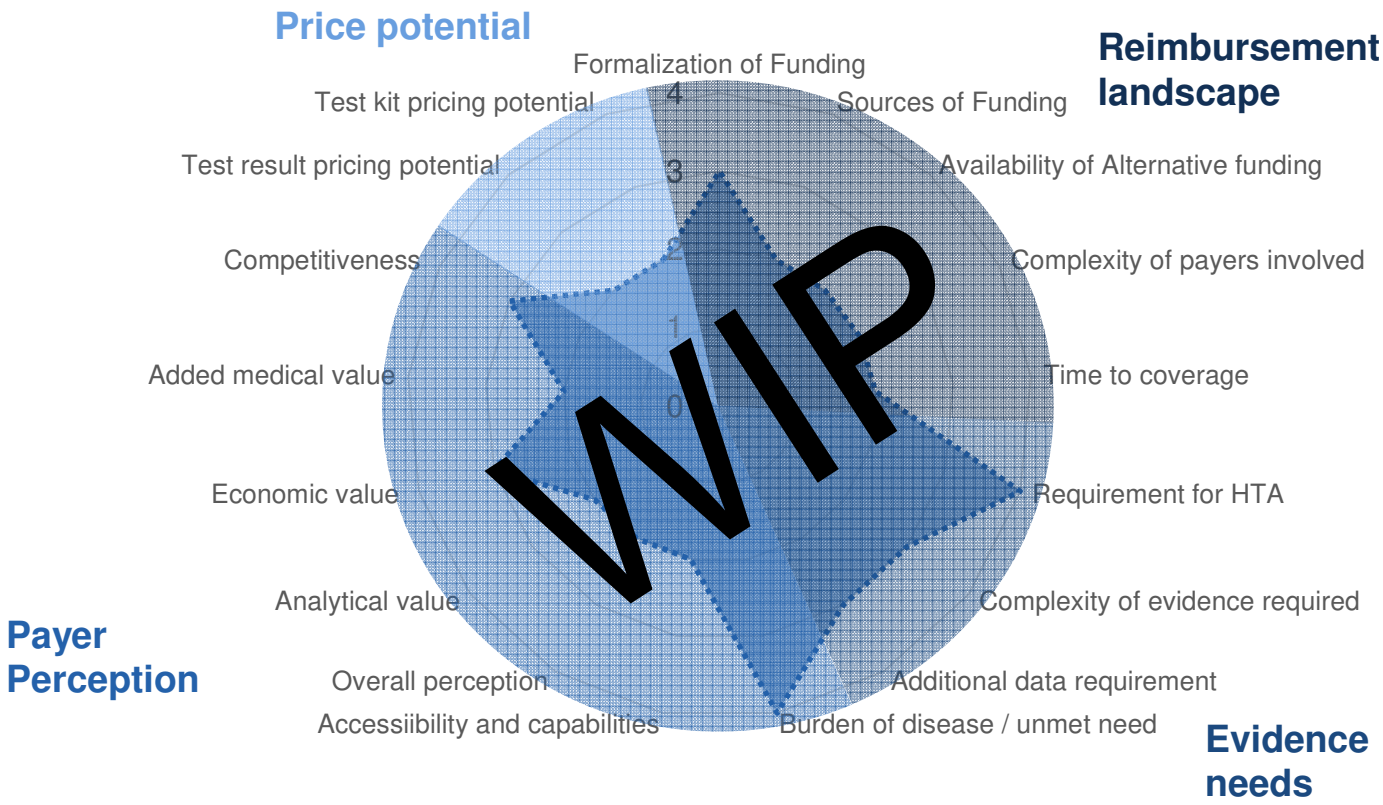
We are currently performing the same assessment for a disease program (hotline, edu/coaching, app) in the area of diabetes



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Key takeaways

- An early MA assessment benefits traditional Personalized Medicine solutions as well as new tools and technologies
- Early MA assessment is particularly needed for PM due to the high level of unknown and the impact these can have on the drugs
- An early MA assessment provides precious insights into critical decisions (evidence, pricing, VP) and early stakeholder engagement activities
- Dx already available on the market and even reimbursed will still have Market Access challenges
- A standard framework allows for an efficient (cheap and fast) and comparable approach across different products and solutions
- Do not expect your Dx / solution partner to drive these activities as often they do not have the necessary resources

I look forward talking to you!

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