Innovative Agreements

CORD 2024

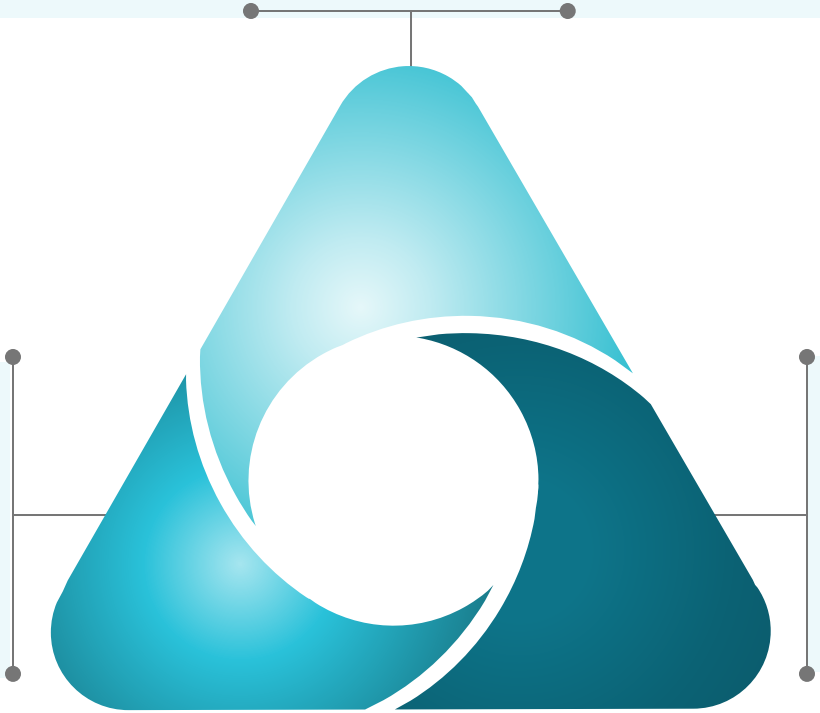
November 26, 2024

MORSE CONSULTING | DECIPHERING MARKET ACCESS

Can we use innovative agreements to facilitate access and time to funding?

Simple Innovative Agreement

Free drug to initiate therapy, with public payers only reimbursing for patients who **demonstrate benefit after the initial trial period**



Performance-Based Rebates

"Performance" can be **clinical outcomes** or something simpler like no **early discontinuation**, ability to **tolerate** therapy, etc.

Other Innovative Agreements

- Expand **TLR & pTAP eligibility**
- Leverage novel infrastructure or registries for **RWE collection** to enable Outcomes-Based Agreements

IA: Innovative Agreement; OBA: Outcome-Based Agreement; RWE: Real-World Evidence

CASE STUDY: Free drug (e.g., through manufacturer Patient Support Program)

Coordinate free drug to initiate therapy as part of an Innovative Agreement

And have payers continue and fund drug for patients who can tolerate and/or benefit

Will need to be a way for patients to access therapy and for the manufacturer to reimburse the drug if patients cannot/do not want to access the PSP

Example: Kuvan (sapropterin)

Sapropterin
Brand(s): Kuvan
DOSAGE FORM/ STRENGTH: 100 mg tablet
Updated December 2, 2020

Ongoing funding of sapropterin (Kuvan) will be considered through the EAP for non-pregnant patients and patients actively planning pregnancy who have a diagnosis of Phenylketonuria(PKU) and who have demonstrated a response to the initial 6 month trial of sapropterin [generally reimbursed through the Biomarin, the manufacturer of Kuvan].

Free drug can be given:

- For first prescription / first cycle etc. (e.g., 1-3 months)

OR

- For the duration of entire initial approval period per CDA/HTA criteria (e.g., 6 months)

PSP: Patient Support Program; EAP: Exceptional Access Program; HTA: Health Technology Assessment

CASE STUDY: Example of a Clinical Outcomes-Based Agreement

Small Patient Population & Treatment In Specialized Centres

- Small numbers of patients & prescribing physicians = easier to follow individual patient outcomes (based pricing at an individual level)
- Pivotal trial's primary outcome was clinically meaningful - measure of value

Outcome data can be captured in various ways

- Most rare diseases are under special authorization - outcome details are provided to drug programs/private payers for ongoing funding
- PSPs or registries could aid in collection of data and/or invoicing

Rebate Based on Outcomes

- Some patients had more meaningful improvements than others
- Different rates of discounts could be set for different response rates
- MFR took on risk that patients would have similar or better benefits in real-world vs. trial

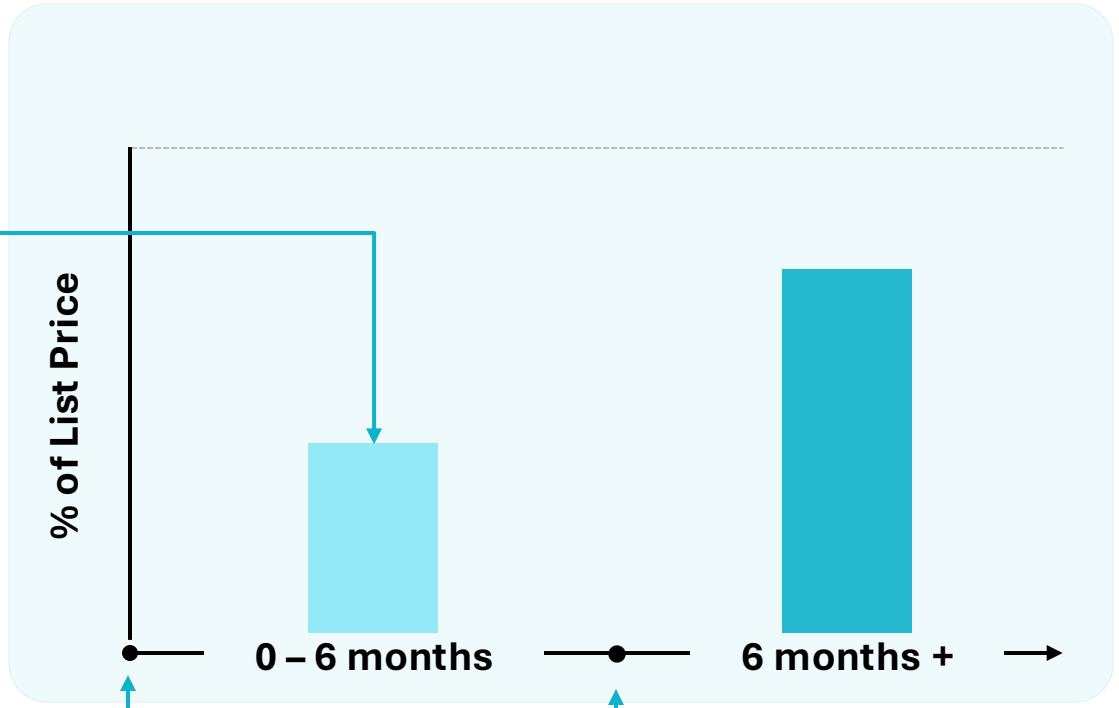
Implementation

- Implementation issue mitigation/exception management must be considered (e.g., what if patients miss follow-up assessments? What if patients move to a different province?)

CASE STUDY: Simple Performance-Based Rebates
("Performance" = able to tolerate/ stay on therapy)

For drugs where long-term use is required for benefit but there is uncertainty around early discontinuation/ intolerance, **higher rebate during highest likelihood of discontinuation**

Potential OBA Agreement Structure



Can be easily adjudicated

Based on how long the patient stays on therapy as an indirect estimate of performance

Quicker to manage risks

Associated with uncertainties vs. having to create a robust RWE study

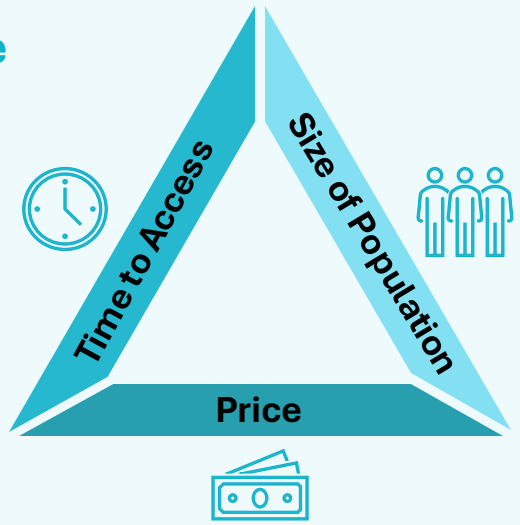
OBA: Outcome-Based Agreement; MD: Doctor of Medicine; PSP: Patient Support Program

WHAT IF: RWE (e.g., leveraging Registry or extension studies, etc.) is utilized to address important gaps in clinical trials after an initial (TLR-like) positive?

Manufacturers could prepare early for pre-NOC submissions and discuss the evidence generation plan and feasibility of addressing potential uncertainties/ gaps with HC & CDA

Time to Access, Size of Population, and Price are Trade-Offs

pTAP will likely require mfr to provide a **lower price during time of uncertainty** in exchange for early access



Initial Submissions

- **RWE** and **extension studies** to address gaps (i.e. population & outcomes not studied)
- Can be **assessed for reassessment by HTA**, especially where clinical trials are not feasible.

TLR & pTAP

- Current TLR & pTAP Process can apply

OPPORTUNITY

Early access, expansion of TLR to DRDs and other drugs where Phase III not possible

RISKS

Mfr will have to **trade price** and take on risks for **early access**; could have ICER thresholds; nothing firmly established yet

TLR: Time-limited recommendation; RWE: Real-World Evidence; DRD: Drug for Rare Diseases