20 SENSE

THE 20SENSE REPORT

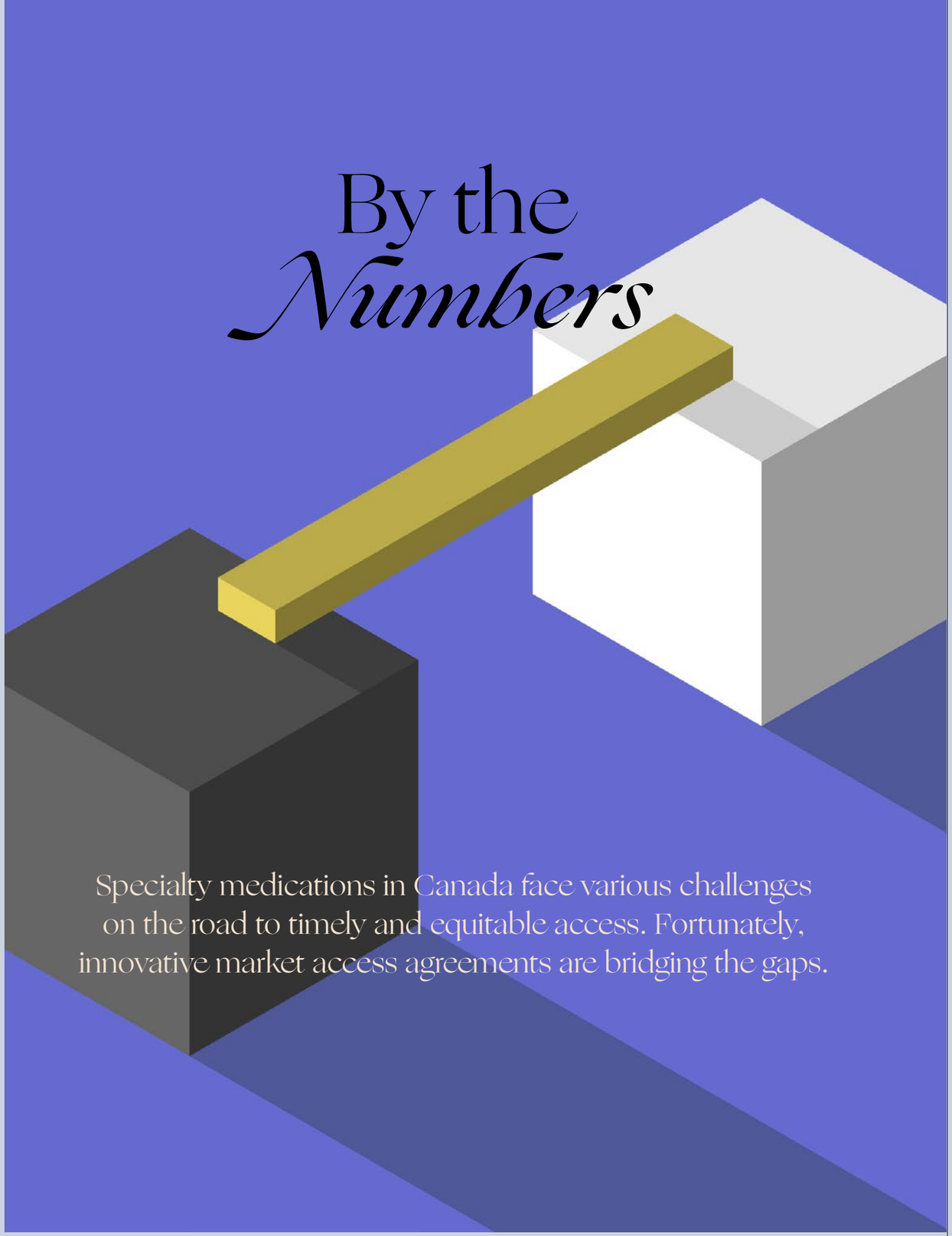
Spotlight on the Canadian Specialty Pharmaceutical Market

April 2021
Issue 16

Outcomes-Based Agreements in Canada

A natural adjunct to specialty drug innovation, outcomes-based agreements are poised to disrupt the Canadian specialty drug reimbursement scene

By the *Numbers*



Specialty medications in Canada face various challenges on the road to timely and equitable access. Fortunately, innovative market access agreements are bridging the gaps.

RACE TO REIMBURSEMENT

53%
Average proportion of approved Canadian drugs that receive public reimbursement in Canada.[1]

21 MONTHS
Average time from NOC to public reimbursement of drugs in Canada.[1]

22 MONTHS
Time from NOC to public reimbursement for orphan/rare-disease drugs.[1]

19 MONTHS
Time from NOC to public reimbursement for oncology drugs.[1]

43 DAYS
Time from NOC to Alberta listing (on a case-by-case basis) of spinal muscular atrophy drug Zolgensma.[2,3]

COMING SOON: NEW DRUGS THAT CALL FOR NEW THINKING

35%
Percentage of drugs in development that target cancer.[4]

40%
Percentage of pipeline drugs in pre-registration phase that target orphan/rare diseases.[4]

5
Number of gene therapies in the pipeline with breakthrough potential.[4]

GLOBAL ACCESS ADVANTAGE

31%
Difference between public coverage in top OECD countries (96%) vs Canada (65%) for drugs that treat unmet needs.[1]

71
Number of innovative contracts in Italy, the global leader for OBAs.[5]

59%
Proportion of US payers that have executed at least one OBA.[6]

ACCESS INNOVATION IN CANADA

16
Number of known innovative access contracts in Canada as of late 2017;[7] the current figure could be significantly higher.

2
Number of OBAs with some publicly available information in Canada.[8]

37
Approximate number of annual diagnoses of SMA in Canada.[9]

5
Approximate number of annual diagnoses of SMA in Alberta,[9] where eligible patients receive coverage for game-changing drug Zolgensma (the most expensive drug in the world[10]) through an innovative access approach.[11]

AREA OF NEED

4,300
Number of Canadian patients with cystic fibrosis (CF) hoping to get access to novel CF medication Trikafta.[12] EU and US patients have been able to access the medication for some time via managed entry agreements.[13]

$300,000
Approximate annual cost of Trikafta.[14]

9.2
Additional years of life that Trikafta could make possible for children born with CF.[15]

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Outcomes-based Agreements: An Overview

The specialty drug industry can hardly keep up with itself. Every month sees new miracle drugs – drugs that stop advanced cancer in patients with specific genetic profiles or that give new hope to patients born with the rarest of diseases – tumble into the market. These drugs, which require Herculean resources and ingenuity to develop, don't come cheap. This leaves public and private decision makers with the difficult challenge of balancing timely access with budget constraints, while patients stand by.

ALL ABOUT ACCESS

In theory it should be simple: drug innovation saves and transforms lives, and patients deserve access to these drugs. But the assessment framework we've inherited from simpler drug days doesn't have the vision and flexibility to handle the new drug development world.

As Andre Vidal-Pinheiro, head of global pricing and access at Takeda, noted in a 2020 webinar,[5] "standard health technology assessment criteria are not particularly kind to orphan drugs." By definition, these drugs serve small and highly specific patient populations, so they seldom generate the large datasets that regulators traditionally require to demonstrate clinical value. Add the high cost of these drugs to the mix and you're left with what Vidal-Pinheiro calls "a recipe for disaster." Manufacturers argue that these medications change lives, while regulators and payers focus on the risks and cost-benefit ratio. The result? Delayed access.

These challenges are playing themselves out in our national backyard. The tumor-agnostic oncology medication Vitrakvi, for example, received a negative recommendation from pCODR's Expert Review Committee (pERC) because the limited clinical data on the drug left the Committee unsure of its advantage over existing therapies.[16] While the manufacturer's resubmission[17] moves through the system, patients continue to wait. The recent approval of Zolgensma[2] – reportedly the most expensive drug in the world at $2 million per year[10] – is compelling governments to think about access in new ways. At over $300,000 per year, the breakthrough cystic fibrosis drug Trikafta, currently undergoing priority review in Canada,[18] will likewise require innovative access solutions to thrive, and the new drug pipeline promises more of the same.

SHARING THE RISK

Outcomes-based agreements (OBAs), also known as value-based, managed-access, or performance-based agreements, are poised to bridge such access gaps. Based on the principle of dividing risk between manufacturers and payers, these agreements typically include both a data-collection component – to better understand real-world efficacy – and a commercial agreement to delineate the risk-sharing terms.

What is an outcomes-based agreement?

An agreement between a manufacturer and a payer in which the manufacturer will issue a refund or rebate to the payer based on how well the therapy performs in a real-world patient population, measured against an agreed-upon, pre-defined set of benchmarks.

A number of countries with single-payer healthcare systems have developed formalized risk-sharing pathways for rare and high-cost diseases. Recognized globally as an OBA leader, Italy has had such arrangements in place since 2006.[19] Several other European countries have followed suit, with OBAs that enable quick listing followed by reassessment based on insights from real-world evidence (RWE).[20]

LEADERS TO LEARN FROM

These regions have shown leadership in the OBA realm and can serve as models for future OBA development and implementation.[8]

Region	Achievement
Australia	Transparency on OBA implementation
UK	Health Technology Assessment (HTA) and payer collaboration on access to high-cost drugs
France, UK, Germany, Italy	Pathways that include options for innovative agreements
Canada (Alberta)	Innovative rapid access solutions
Italy	Most number of OBAs established

THE CANADIAN OBA LANDSCAPE

While several steps behind these jurisdictions, Canada has recognized the need to take action. To help Canadians with rare diseases access the drugs they need, the country plans to invest up to $1 billion over two years into a national strategy that includes pay-for-performance and risk-pooling models[21] – in other words, OBAs.

Based on the findings of a 2017 global report, Canada already had 16 publicly disclosed "innovative contracts" at the time,[7] and the number has undoubtedly grown since then. While the confidential nature of OBAs has constrained the free exchange of information about these agreements, speakers in public forums have confirmed the existence of OBA-style schemes in Canada for a handful of specific drugs, such as Spinraza and Revestive.[5,8]

An OBA by Another Name – Spinraza's Story

In a virtual conference organized by the Canadian Organization for Rare Diseases (CORD), Quebec-based SMA patient advocate Catherine Boivin alluded to "a type of managed access approval" for Spinraza,[5] suggesting an OBA-style agreement. What steps might have led to this development?

It all began in December 2018, when Quebec's INESSS announced its recommendation to list spinal muscular atrophy (SMA) medication Spinraza on the "promise of therapeutic value," with the possibility of delisting it in the future if real-world evidence shows insufficient benefit. Shortly thereafter, an update to the Saskatchewan formulary explained that patients seeking continued coverage for Spinraza "will be required to undergo ongoing assessment to monitor for improvement over time and must meet renewal criteria for continuation of treatment." Ontario soon joined Quebec and Saskatchewan in broadening criteria for Spinraza access to include adults "on a case-by-case basis." London, Ont. pediatric neurologist Dr. Craig Campbell applauded the agreement, stating it would allow the SMA community to "collectively document the long-term real-world data to further demonstrate the positive impact of [Spinraza.]"[24] It seems these provinces parlayed pCPA [pan-Canadian Pharmaceutical Alliance] negotiations into an agreement about how to pay for Spinraza – an agreement that looks a lot like an OBA.

WHEN THE SHOE FITS

Listing decisions depend on a web of interconnected factors, which in the case of novel medications include some gaping unknowns. This may help explain why only 20% of medications assessed by the UK's National Institute for Health and Care Excellence (NICE) received a final recommendation after a single committee meeting. A review of NICE's assessment process attributed this sluggish performance to "mischaracterization of a technology's value and effectiveness by manufacturers, leading to uncertainty of doubt surrounding its cost-effectiveness following independent academic review."[26] The good news: in the majority of cases, "decision optimization" involving enhanced commercial agreements [read: OBAs] removed the barriers to a positive decision.[26]

This is not to say that OBAs make sense for all specialty drugs. In fact, as advised by the UK's 2021 Commercial Framework for New Medicines, complex arrangements such as OBAs should "only be considered once simple discounts (which facilitate fastest access) have been demonstrated to be unsuitable."

OBAs offer the greatest benefit in the following circumstances:

- **Variable response:** When clinical trials suggest that only a limited proportion of patients (e.g. 50%) reach a desired health outcome, OBAs can reduce a payer's risk by limiting ongoing reimbursement to patients who meet agreed-upon outcome criteria.
- **Limited data:** When promising but incomplete early clinical trial data makes it difficult to assess a drug's performance, an OBA can provide access to patients with no treatment alternatives to meet their needs.
- **Stalled negotiations:** When the drug manufacturer and the HTA and/or payer disagree on the magnitude of therapeutic benefit suggested by clinical trial data, an OBA can help patients access treatment earlier, with continued access dependent on proof of benefit.

FROM INTENTION TO IMPLEMENTATION

Manufacturers and payers who cut their teeth on simple financial contracts may wonder how to get the OBA ball rolling – and with good reason. As market access expert Dr. Philip Spearpoint asserted in a talk on managed entry agreements in the EU, "if you think agreeing to an OBA is difficult, wait until you get to the implementation."[29] The biggest challenge? Devising a framework that guides all stakeholders toward an achievable, mutually satisfactory agreement.

Fortunately, future-oriented stakeholders are working on strategies to enhance the opportunity for OBA implementation in Canada. Alberta's Institute for Health Economics is creating tools and resources to support risk-management agreements and other innovative funding options.[30] The Canadian Centre for Applied Research in Cancer Control is developing a framework for using RWE to support oncology drug funding decisions, with support from the Canadian Institute for Health Research under the CanREValue initiative.[31] And for the RWE & OBA Working Group, co-creating implementation solutions with Canada's OBA stakeholders tops the priority list.

From the manufacturer perspective, the success of an OBA rests on good planning – ideally with a cross-functional OBA team to take the lead on negotiation and implementation. After confirming a drug's suitability for an OBA, the team must ensure that both parties at the negotiating table – manufacturer and payer – agree on the outcomes of interest and how to measure them.[28] To avoid unpleasant surprises down the line, the negotiated agreement should cover all aspects of the OBA – from patient consent and follow-up to data collection, reporting and adjudication.

To close the loop, manufacturers can share their OBA learnings with the industry, as did Pfizer in a 2017 public document describing the reporting requirements and pricing conditions of its Australian managed entry agreement for the lung cancer drug Xalkori.[32] Such transparent sharing, which need not involve disclosure of confidential information, helps move the OBA space forward for all players.

There is a lot to celebrate at this juncture: OBAs have entered the public conversation, implementation frameworks are on the design table, and early adopters are making their mark. The industry now needs to move forward with customized OBAs that reflect the specific challenges of each drug, delineation of roles and responsibilities, as well as mechanisms to share OBA practices so the space can mature.

A fuller integration of OBAs into the specialty drug ecosystem will require creativity, collaboration and courage. By all indications, rare-disease and precision oncology drugs will lead the charge. Patients are counting on it.

OBAs have entered the public conversation, implementation frameworks are on the design table, and early adopters are *making their mark.*

OBA Frameworks

OBAs need to offer a degree of certainty in a landscape riddled with unknowns.

They also need to integrate with HTA, payer processes, and commercial strategies – to "speak the same language," as it were.

LIST NOW, REVISIT LATER

In the EU, countries with well-developed managed access pathways, such as France, Germany, Italy, and the UK, manage uncertainty by requesting RWE and using it to reassess a drug's value.[20] The model follows more or less this sequence:

- HTA assessment
- Creation of an OBA to enable quick listing, with built-in requirement for RWE generation
- Agreement to the commercial terms and pricing of the drug within the OBA
- Collection of RWE
- Analysis of RWE to reassess the drug's health-economic value
- Price adjustment as necessary, and closing the OBA

Such a model can only work if the health-economic evaluation complements the initial clinical assessment of the medication, rather than competing with it – one of the principles outlined in the UK's Commercial Framework for New Medicines.[27]

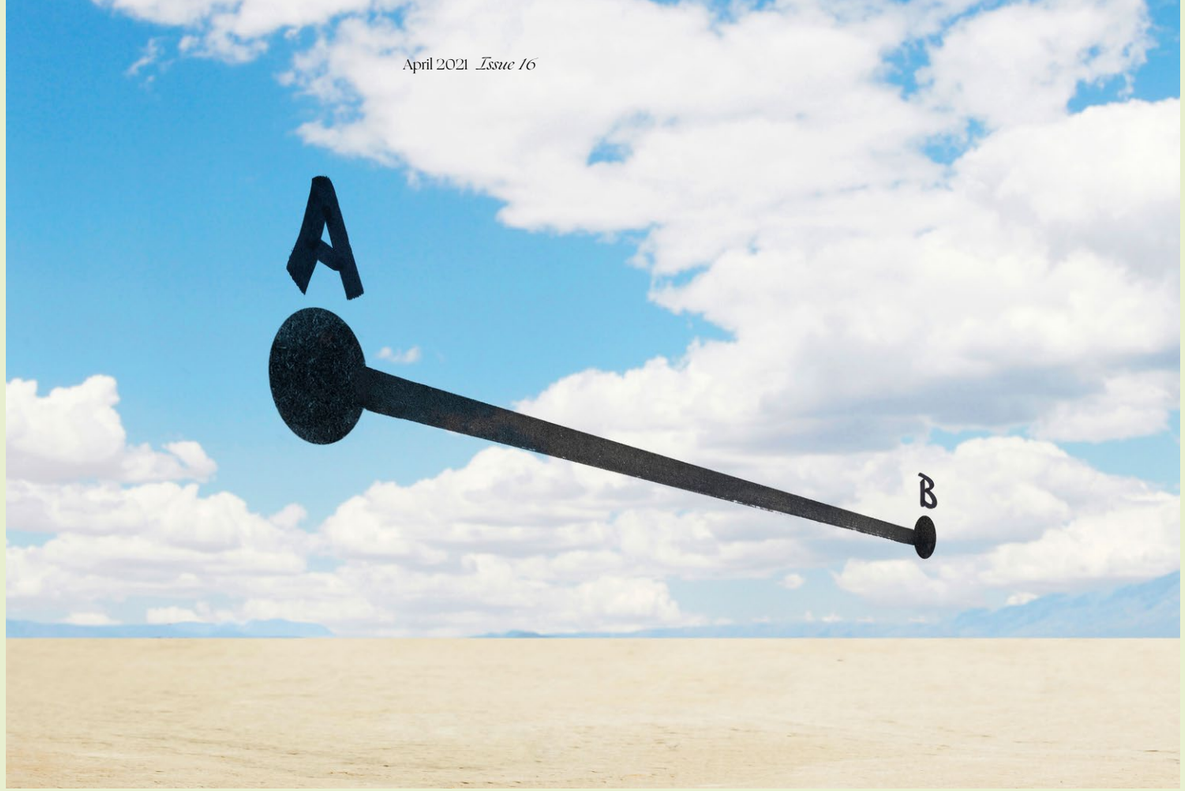
NATIONAL AND PROVINCIAL LEADERSHIP

Though not quite as far along the path as Europe, Canada is taking steps to align its HTA process with commercial realities – what Suzanne McGurn, president and CEO of CADTH, refers to as "health technology management over the lifecycle."[33] In a recent podcast, McGurn noted the "opportunity to bring [this life-cycle approach] forward as part of what CADTH does." This will involve "figuring out which are the right things to go back and look at."

The province of Quebec has embraced a similar vision in its 2017-2027 Life Sciences Strategy, which counts faster access to promising drugs and support for RWE projects among its objectives.[34] Recent HTA recommendations by INESSS [Institut national d'excellence en santé et services sociaux] confirm that the province is walking the talk. These recommendations generally follow the model previously described – accelerated recommendation, followed by RWE generation and reassessment – as exemplified by Galafold, a drug for Fabry Disease. Assessed in late 2018, Galafold represents the first instance in which INESSS evaluated a drug based on a promise, rather than definitive proof, of clinical value.[35] Just a month later, INESSS recommended listing the spinal muscular atrophy (SMA) drug Spinraza on the condition of RWE generation and clinical follow-up, with the possibility of delisting at a future date if the data fails to demonstrate clinical value.[36]

INESSS took a similar route with another game-changing SMA therapy, Zolgensma, in recommending an initial listing followed by reevaluation of the drug within 3 years based on real-world data collected by the manufacturer.[37] CADTH followed suit with a positive recommendation in March 2021, though with more "classic" eligibility criteria and a request for a price reduction.[38] Zolgensma manufacturer Novartis had evidently hoped for more, as reflected by its community statement expressing both satisfaction with the positive recommendation and disappointment that it is "limited by age, without a mechanism for case-by-case review."[39]

While the provinces typically wait for HTA recommendations and pCPA negotiations to be complete before proceeding to drug listing, Alberta decided Zolgensma called for a new approach: the province listed the drug on a case-by-case basis just six weeks after Health Canada approval.[40] "There is no budget cap – we are focused on kids not falling through the cracks while the regular review processes are ongoing,"[41] said Alberta Minister of Health Tyler Shandro in a Jan. 2021 announcement, giving families affected by SMA new reason to rejoice. As SMA parent Lana Bernadin put it, "we feel a great sense of joy that no other family will be



faced with raising money for the world's most expensive medication."[11] While Ontario has yet to announce a formal program to cover Zolgensma, the province stated it would authorize it on a case-by-case basis after a family launched a GoFundMe page to help pay for the drug.[42]

Record time

The province of Alberta raised the access bar to new heights when it listed Zolgensma, the world's most expensive drug and a game-changer for patients with spinal muscular atrophy, just 6 weeks after Health Canada approval.

This approach lines up with the European access program for Zolgensma, Day One, which runs ahead of the HTA process to speed up access and gives payers outcomes-based rebates along with the option to defer payments to manage budget impact.[43] From the manufacturer's perspective, the collection of RWE before pricing negotiations can bolster the value story and alleviate payer concerns about cost-benefit.

Done right, OBAs promise wins for both manufacturers and payers – and above all, for patients.

What Canada can learn from the UK

A peek at the UK's drug appraisal and commercialization strategy reveals some forward-thinking developments. In the oncology realm, the Cancer Drugs Fund (CDF) marries the complementary objectives of speeding up access to cancer medications and giving pharma companies a fast track to NHS funding.[44] Reformed in 2016 to curb the approval of cost-ineffective treatments, which had increased by £185 million in the previous year, the CDF focuses on drugs for which ongoing data collection can resolve clinical uncertainty.[26]

More recently, in its 2021 **Commercial Framework for New Medicines**, the NHS set out broad principles to bring clinical assessments and commercial negotiations into alignment, including "avoiding burdensome data collection and disproportionate additional cost" in OBA-type arrangements.[27] The managed access agreements (MAAs) described in the document give pharma companies a mechanism to "offer a value proposition at or below the lower end of the standard NICE cost-effectiveness threshold range."[27] Key components of these MAAs include a data collection agreement to mitigate clinical uncertainty and a commercial access agreement. This high-level coverage of commercial options for complex drugs make the document a unique source of learning.

The OBA value proposition rests on the collection and evaluation of real-world data (RWD) and real-world evidence (RWE).

In theory, the data can spring from virtually any source as long as the manufacturer and payer agree on its validity – and keep administrative complexity to a minimum. As a leading expert advised in a presentation on managed entry agreements in the EU, **"keep criteria for analysis as simple as possible to avoid paralysis."**[29]

Overall survival, arguably the simplest metric of all, brought clarity to the Australian data collection program for Xalkori, a treatment for patients with a subset of non-small-cell lung cancer. The program supports the managed entry scheme set out for the drug, which requires the manufacturer to rebate a pre-specified percentage depending on 12-month survival rate.[32]

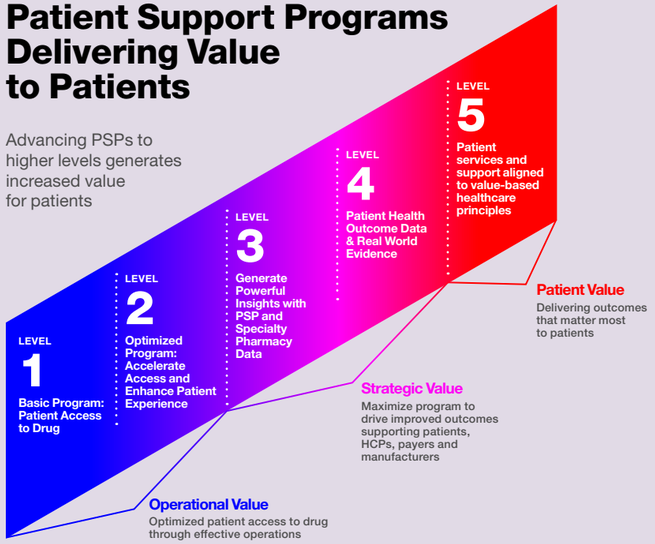
The UK's NICE also includes survival, along with ventilation and respiratory events, motor function, and scoliosis surgery, in its data requirements for the spinal muscular atrophy (SMA) drug Spinraza.[45] In the interest of preserving data integrity, physicians are required to enter specified clinical data into the SMA REACH registry. The agreement also mandates the collection of patient-reported outcome measures.

A HIDDEN CANADIAN DATA GEM: THE PATIENT SUPPORT PROGRAM

Recent months have seen patient support programs (PSPs) emerge as a prime candidate for OBA data collection in Canada. At Market Access Canada's 2020 summit, AstraZeneca Canada Vice-President Mo Amin highlighted the opportunity for PSP patient data to support access.[46] Similarly, AstraZeneca's Dr. Michael Seewald flagged PSPs as a fitting source of Canadian OBA data and a vehicle for understanding the patient experience in a recent conference on RWE methodologies.[47] Building on the theme, a Pfizer presentation at the 2020 CAPT conference positioned PSPs as a potent source of RWE and a bridge between the clinical assessment and lifecycle management of a drug.[48]

In fact, PSP-based data collection is well underway in Canada. All PSP vendors who responded to a 2020 Canadian PSP data capabilities survey build data collection into their infrastructure, and all but one surveyed manufacturer receive data from their PSP vendors.[49] Most importantly, close to half (47%) of manufacturers have used the data for HTA analysis. By all indications, Canadian stakeholders are gaining experience in harnessing PSP data to support market access and reimbursement – and plan to continue to invest in this mechanism.

PSP data stands to benefit all stakeholders. While even basic PSPs help patients gain access to medications, building real-world and patient-reported data collection into a PSP can yield insights that improve outcomes and support OBAs. The insights can feed into the PSP itself in a cycle of continuous improvement, culminating in a program that offers the services that matter most to patients and the best value for all parties.



PUTTING PSPS TO WORK

There is no formula for creating a PSP. Designed to meet specific clinical, market and patient needs, they differ widely in the patient services and data capabilities they offer. From the OBA perspective, this flexibility has a big upside: manufacturers and PSP service providers can plan for the data-collecting mechanisms needed to support a particular OBA. At the same time, some payers may have concerns about data collected within a manufacturer-sponsored program.

Does this concern have merit? And why turn to PSPs for data in the first place? Below, leading Canadian data and PSP experts weigh in.

- **Tara Cowling,** Director and Managing Principal, Medlior Health Outcomes Research, **on the benefits of using PSP data over other administrative datasets:** "PSP data is typically national-level, longitudinal, with clinical confirmation of included patient populations, which is why they are suited to examining specialty drugs. Some of the data collected from PSPs – patient-reported outcomes, caregiver-reported outcomes, and measures of disease progression, for example – are not routinely captured by health system datasets, which can also help evaluate the cost-effectiveness of a treatment."
- **Kimberly Fougere,** Associate Director, Offerings Management, STI, **on the power of PSP data:** "It allows us to provide insights to the manufacturer on patient adherence, payer landscape, and other parameters. Having these discussions before the PSP is designed ensures we can build in the desired data requirements. We take data privacy seriously and follow confidentiality guidelines sourced from Canadian privacy laws."
- **Kelly Isaacs,** Vice President, NavieGo Patient Programs, BioScript Solutions, **on the efficiency and cost-effectiveness of PSP data:** "Data from PSPs leverages the existing infrastructure. The ability to collect information directly from patients and physicians is invaluable. When designing a PSP, we work with clients to decide on what data to collect and how to do it. And the earlier it happens in the planning process the better the data can meet the needs of all parties."
- **Taflyn Hornibrook,** Co-CEO and Head of Patient Programs and Stakeholder Relations, Sentrex Health Solutions, **on the power of PSP data to support OBAs and help all stakeholders:** "It seems natural to take PSPs a step further and collect data for an outcomes-based agreement. There is an opportunity to define upfront what success looks like for all parties involved in a PSP – patients, physicians, manufacturers, and payers."
- **Remi Menes,** Vice President, Specialty Patient Programs, McKesson Canada, **on the value of PSP data to manufacturers and payers:** "PSPs are designed to gather the data points needed to answer key questions about the patient journey, which is very helpful to the manufacturer. PSP data could allow the payer to see how the drug is used and what value it offers. Ideally, to support OBAs, payers would be engaged in the upfront PSP data design process."
- **Jodi Adams,** Director, Business Intelligence and Transformation, SDM Specialty Health Network, **on the opportunity for PSP data in the digital world:** "The increasing role of digital channels to connect with patients and caregivers represents a chance for PSPs to collect more targeted information. By working within a PSP framework, payers can gain better access to patient-reported outcomes and real-world data to support coverage decisions."
- **Sean McBride,** Director of Commercial Operations, Bayshore Specialty Rx, **on the challenges and opportunities for real-world data collection:** "Real-world data can help answer clinical questions that the trial setting cannot. At the same time, the integrity of the data depends on how it is sourced, collected, and validated. To get the most out of the data, it is important to identify and address inherent gaps or biases that may impact the outcome, and ensure consent and privacy are addressed."

In brief, the power of data lies in its reliability, which in turn depends on training the right people to do things the right way. Diamonds in, diamonds out. Whether generated from PSPs or another source, a strong data program builds trust between manufacturers and payers. And trust is the commodity OBAs depend on more than anything else.

The Italian Connection

Luca Pani on the need for more creative market access agreements – and what Canada can learn from Italy

A globally recognized expert in pharmacology, regulatory and access strategies, Dr. Pani served as CHMP and SAWP Member of the European Medicines Agency (EMA) and Director General of the Italian Medicines Agency (AIFA) from 2011 to 2016. During his tenure at AIFA, he revolutionized the agency's approach to drug approval, pricing and reimbursement, elevating Italy to an international leadership position in the use of managed entry and outcomes-based agreements. The co-author of hundreds of scientific papers, Dr. Pani currently holds academic positions at the University of Modena and Reggio Emilia in Italy and the University of Miami, handles regulatory strategy and market access innovation for the influential and technology advanced drug development consultancy VeraSci in Durham, NC, and is arguably the leading global expert on outcomes-based agreements.

Among the disruptive changes you introduced at AIFA, is there one that was especially meaningful to you?

AIFA was the payer who negotiated access for Strimvelis, the world's first gene therapy. This is for people who can't produce white blood cells and thus have to spend their lives isolated from infectious agents – in a bubble, as it were.

We got the drug listed within 55 days of the dossier request for regulatory evaluation at a price of 594,000 Euros, with reimbursement contingent on results and clear outcomes measurements to guide us.

Did you get support for your approach from the Italian government?

Yes. Our parliament has a law that mandates offering treatments to patients when no reasonable alternatives exist. We also have web-based certified registries which are legally binding in the context of a drug pricing negotiations. Having binding laws, as opposed to mere guidelines, is the key to empowering successful negotiations and timely access, especially for high-cost therapies.

How do registries fit into the market access framework?

Registries define the population (even in sub-strata if needed) with precise inclusion and exclusion criteria and what we call the "value endpoints" and can be used to compare a new treatment to the standard of care. Data from registries are the "fuel" for market access agreements. Most agreements for expensive, life-changing medications requires proof of duration for the clinical response within 2-3 years, which registry data can help establish.

Dr. Luca Pani

Early access first, formalized reimbursement criteria later – we'll be seeing a lot more of this model in the future with innovative life-saving therapies.

Duration of response is an obviously important decision-making point. Who bears the burden of demonstrating a lasting response?

Nowadays it's up to the market access professionals to provide the data on duration of response, though access to a medication cannot be held hostage to this evidence when it is limited. You can't tell a parent you won't give a medication to their dying child because you don't know how long it will work. Because so much is at stake for families, regulators may even approve early access for a larger population than that proposed by the manufacturer, as was the case with Zolgensma in Europe. Early access first, formalized reimbursement criteria later – we'll be seeing a lot more of this model in the future.

What else can manufacturers do to expedite access and set fair pricing?

Non-inferiority data can help a manufacturer get a listing, but payers will understandably expect to see superiority data, with clinically relevant endpoints such as overall survival, to justify a high price. High-quality health-economic data can also strengthen a case. For example, if you can demonstrate that a novel combination treatment prevents the progression of, say, hepatic fibrosis to hepatocarcinoma and reduces the need for costly liver transplants, you can leverage these downstream certifiable savings in pricing negotiations. The problem is that the drug procurement and transplant departments in a healthcare system often operate in silos. We need a One Health model approach, with all departments talking to each other.

We've seen incredible innovation in specialty medicine in the past few years. How has this innovation changed the regulator's role?

Today, regulators are not expected merely to keep 'bad medicines' off the market. They must align drug licensing with patients' needs by granting them timely access to promising new technologies. Of course, quick access comes with a burden to prove effectiveness. This entails more stringent monitoring and data generation throughout the life cycle of the medication.

What would you recommend to help Canada move forward in the OBA space?

I would suggest building an early access scheme, ideally with legal teeth. There should be a centralized body giving binding recommendations on reimbursement criteria, with some additional negotiating room at the provincial level. Regulators and payers need to get together to make it happen.

How important is it for industry to explore innovative market access agreements, such as OBAs?

It's critically important. Specialty medicine innovation isn't going away, and affected families won't put up with protracted negotiations when a loved one's life hangs in the balance. Payers won't be pushing for more creative negotiations – they're used to thinking in terms of caps or discounts – so it's up to drug manufacturers to take the lead.

Should manufacturers be sharing their OBA experience more transparently within the industry?

We can't expect manufacturers to reveal pricing details – and we don't need to know the specific figures. Transparency in OBA implementation processes, however, is much more valuable for shared learnings.

How important is the quality of the data to support OBA-type agreements?

Payers are generally willing to work with real-world data, as opposed to regulatory-grade data, as long as the integrity of the data is assured. Some manufacturers are even venturing into creative new data collection territory. For example, Biogen recently announced a partnership with Apple to investigate how iWatch and iPhone could help in screening and monitoring possible declines in cognitive health.

Has the COVID-19 pandemic taught us anything that can be applied to innovative market access agreements?

The pandemic has shown us that things previously thought impossible are doable. Imagine if we had been told a year ago that over 600 million people could be quickly vaccinated under emergency use authorization – nobody would have believed it. Now that we know how fast and effectively medical science can intervene, it will be harder to justify requesting, say, 2 years of additional safety data before making a life-saving drug available to patients. Science will dictate speed, and early access is the new target. Patients are demanding it and rightly so.

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On the reading *list*

Innovative pharma contracts: When do value-based arrangements work?

Ontario to cover cost of drug for rare neuromuscular disease on a 'case-by-case basis'

Canada's RWE and OBA Working Group: 2020 Research and Outputs Executive Summary

NHS commercial framework for new medicines, Feb. 2021 update

Use of real-world evidence in cancer drug funding decisions in Canada: a qualitative study of stakeholders' perspectives

The endless frontier? The recent increase of R&D productivity in pharmaceuticals

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11 Families of Alberta children suffering from spinal muscular atrophy (SMA) may now be eligible to receive funding for gene replacement therapy treatment. Cure SMA news release. **https://bit.ly/3rRZCEK**

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