# THE 20SENSE REPORT

SPOTLIGHT ON THE CANADIAN SPECIALTY PHARMACEUTICAL MARKET

JANUARY 2019_ISSUE 7

20SENSE

Making sense of Canada's specialty pharmaceutical market

## Wanted: More and Better Specialty Data

**Fact:** specialty medications are expensive. **Fact:** more of these medications enter the market every year. The innovation is to be applauded, but the status quo can't be sustained.

**Solutions?** This is where it gets tricky. Stakeholders have been trying various approaches, but costs keep rising and evidence lags behind funding decisions. And that's not fair to patients, prescribers, manufacturers, or payers.

There's an elephant in the room and it's called data. Specifically, we need more data on how specialty drugs perform in the real world. We can then aggregate the data into real-world evidence (RWE) that lays bare a drug's real value to patients, enabling us to make the tough calls on which treatments to develop, prescribe, and pay for.

Randomized controlled trials (RCTs), as currently designed, serve an important purpose: the data they generate allows regulators to decide which drugs to approve. The problem arises when stakeholders attempt to use the data beyond its natural boundaries.

Patients in clinical trials are hand-picked to meet specific and narrow criteria:[1] they must have a BMI between X and Y; have diabetes but no heart disease; be free of comorbid conditions requiring medication, and so on. In the real world, patients come in all shapes and sizes, have several medical conditions, and have patient support programs and apps to help them manage their medication regimens. Off-label prescribing adds a further layer of complexity to the clinical picture.

In such real-life scenarios, a drug may perform less reliably than RTC results might suggest. In an analysis of 21 IV cancer therapies involving over 8,000 Ontario patients, for example, population-based studies yielded significantly poorer survival and toxicity outcomes than clinical trials.[2]

In another real-world study, side effects and suboptimal efficacy led 44% of patients on a first-line leukemia drug – a drug intended for long-term therapy – to switch to another treatment within 3 years.[3] On the flip side, some medications show greater muscle in the real world than expected from clinical trials.

We need more – a lot more – data of this type. Much of the real-world data being generated today does not exist in a form stakeholders can effectively use. The data may not apply to the patient scenarios most useful to clinicians and payers. Also missing are the processes that would allow stakeholders to access, interpret, and validate the data so it earns the required trust.

The scarcity of actionable real-world data sets back manufacturers, payers, physicians, and above all patients. Absent data on how a specialty drug performs in a particular type of patient, a prescriber is left to make educated guesses. She prescribes →

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treatment A, waits three months, and when the patient doesn't improve, switches to treatment B, C, and D – finally stumbling on the door with the prize behind it. Real-world outcomes data could have helped her select the winner first.

With the rumblings of major reforms to contain drug-spend – the Patented Medicine Prices Review Board proposal and national Pharmacare come to mind – the data gap raises louder alarm bells than ever. Only with robust, high-quality data can we create equitable reforms that benefit all stakeholders in specialty drug treatment.

On an encouraging note, policymakers have shown a collective will to bridge the gap. For example, the provincial/territorial Expensive Drugs for Rare Diseases (EDRD) working group has set itself the goal of using more real-world evidence (RWE) to evaluate specialized and complex drugs.[5] In the realm of rare disease and cancer drugs, Health Canada and CADTH are working to use RWE more judiciously in the assessment of these medications – and to offer guidance on RWE generation to manufacturers.[6] Then there's the Alberta Real World Evidence Consortium, which seeks to strengthen the province's RWE ecosystem.[7] Even the Pharmaceutical Advertising Advisory Board (PAAB) plans to embrace RWE as a form of evidence.[7] In a proposal to amend its Code, PAAB describes four categories of real-world data, including "market dynamic data" to support claims of drug persistence and switching.[8]

These initiatives offer encouragement, but we have a way to go. We need to move beyond our current fragmented, catch-as-catch-can system and create a RWE-generating infrastructure for specialty medicines. We can begin by leveraging the data that already exists so it becomes a tool for better and more cost-effective patient care.

**All the pieces are in place:** with a few bold moves by manufacturers and payers, and cooperative dialogue among stakeholders, 2019 can be the year we pull RWE from the ivory tower down to earth.

## Unlocking the combinations

Prescribed responsibly, combination therapy involving specialty drugs often works well and may even advance medical science. At the Princess Margaret Hospital (PMH) in Toronto, for example, the Tumor Immunotherapy Program is exploring different combinations of immune-based therapies to mount a stronger attack on cancer.[4] This forward-thinking approach cements PMH's reputation as a cancer treatment pioneer.

In many cases, however, data on combination therapies for chronic diseases stays within the walls of a particular treatment centre. What if we developed a broader, more systematic approach to collecting real-world data on combined specialty treatments? What if we could share the data throughout regions, nationally, and even beyond? Imagine the benefit to industry stakeholders – and above all, to patients.

## The perils of automatic pilot

**Here's a typical scenario in the Canadian specialty-pharma landscape:** a payer works out a deal with a manufacturer based on a drug's performance in RCTs and on Health Technology Assessment (HTA) recommendations. Lacking information on the medication's effectiveness in real-world scenarios, the payer is left to cover the drug, year after year, while patients may miss opportunities for better treatments. This makes as much sense as, say, paying for 64-speed Internet without knowing whether you're actually receiving the service. Perhaps the speed has gone down to 32, or even 16. How would you know? That's where data comes in. Only through systematic use of real-world data will the industry stay healthy as it matures.

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# Insights from a Veteran: A Chat with Helen Stevenson

In 2011, after years of working as a healthcare consultant abroad and then as Executive Officer and ADM for the Ontario government, **Helen Stevenson** founded Reformulary Group, an expert-led health care company focused on helping Canadians make informed choices related to medication and medical cannabis, and helping companies ensure plan sustainability. In this exclusive interview with 20Sense, Helen talks about the need for real-world evidence to drive the use and coverage of specialty pharmaceuticals.

**Q: Can you fill us in on what Reformulary does differently and how you add value?**

We work with a core team of independent experts, including physicians and pharmacists, to review the evidence and create a single managed formulary for private payers. Because we work for employers, our analyses consider not only efficacy of particular drugs, but also treatment strategies to keep people out of hospital and boost productivity.

**Q: Specialty pharmaceuticals are the fastest-growing drug category in Canada. How would you characterize the challenges facing this category?**

In a word, sustainability. As we all know, specialty drugs are expensive. None of the new drugs for cancer, for example, would meet our historical standards for cost-effectiveness. On the other hand, we can't keep patients from new treatments that markedly extend life. So what do we do? We believe the answer lies in creating innovative funding agreements and obtaining real-world evidence to guide decision making.

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**Q: What types of data should we be collecting?**

In addition to tracking treatment success, we need to know how a drug is being used. For example, we may learn that patients are using a costly new drug as an add-on, rather than a replacement for other treatments. We need to track not only the performance of individual drugs, but of treatment combinations and of patients progressing among different therapies.

Real-world data also tells us whether patients are adhering to treatment. With most drugs, adherence typically falls off drastically after the first few months. With specialty drugs, adherence rates tend to be somewhat higher, but side effects and drug treatments for concomitant conditions can act as barriers.

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**Q: We sometimes talk about data vs insights. How is Reformulary approaching insight generation with specialty pharmaceuticals?**

Data is the what, insight is the why. We have been asked to look at patient behaviours for specific diseases, such as multiple sclerosis. What do patients typically use first line? Fifth line? Do they cycle through many different treatments? Using encrypted claims data, we have been able to follow patients' prescription patterns over time. Separately, we mine claims data to identify health conditions within a plan population and then project the impact of pipeline drugs on health plans. Our next step will be to capture real-world outcomes directly. →

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The insights are the factors informing patient behaviours, which could range from access to coverage to market dynamics. We have been encouraging companies to use this intelligence to make business decisions about existing and pipeline drugs.

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**Q: The pharmaceutical industry is currently undergoing reforms, with specialty and rare-disease drugs at the centre of the discourse. Can you comment on these developments?**

Developments such as National Pharmacare could have varying impacts on the pharmaceutical industry. One of the more manageable ideas is a minimum formulary of drugs that would be covered automatically, whether through private plans, public plans, or other forms of subsidy. Plans would be required to cover drugs on the minimum formulary and could cover other drugs at their discretion. As for rare-disease drugs, I am a strong advocate for a national plan for these medications. In my previous role, I learned of families who moved to a different province to get better coverage for these drugs. We need a better solution than that. At the same time, we need to factor in affordability and sustainability into our drug plans and incorporate a system to collect and aggregate real-world evidence to help inform coverage decisions.

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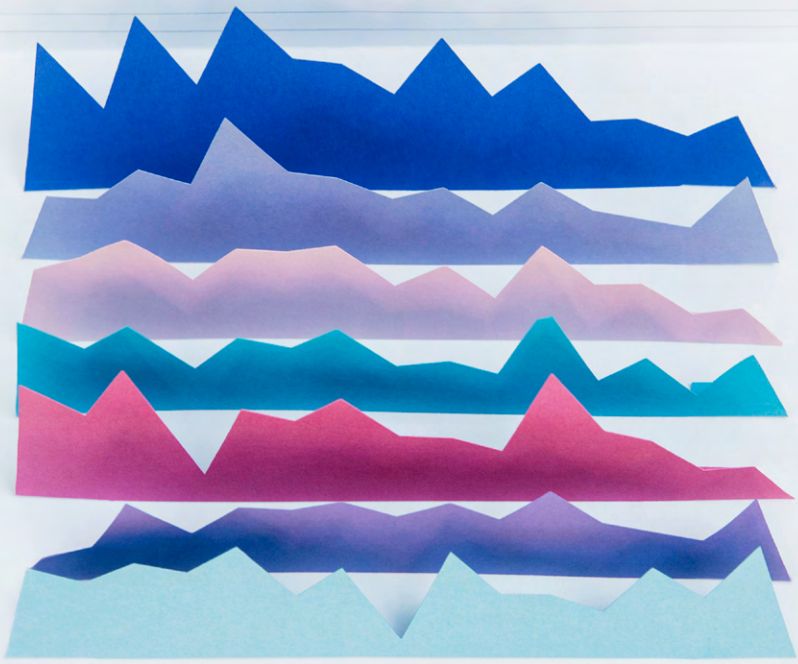
**Q: What advice about data and evidence would you give to specialty pharmaceutical stakeholders?**

It's important to access and use high-quality data. We firmly believe that using actual, unprojected claims data gives the most robust and fullest picture of current and expected usage patterns. When you're launching a new product, real-world data and insights can help you understand the competition and enable you to adjust your strategy accordingly. If you're a payer, you need to know how your clients are using a drug and how it's working for them. You need data to make informed decisions.

**"It's important to access and use high-quality data. We firmly believe that using actual, unprojected claims data gives the most robust and fullest picture of current and expected usage patterns."**



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# Untapped Sources of Data, Evidence, and Insights

**Specialty pharmaceutical data has many faces. It can track clinical outcomes, explore competitive pricing strategies, and zoom in on prescriber habits, to name just a few. We've already established the why of collecting it: to support best practice for prescribers, fair pricing decisions for payers, and better access for patients. But where do we get it, and how do we ensure all parties buy in?**

As it happens, the specialty drug ecosystem has the people and processes required for meaningful, disease-specific data collection. Observational studies, compassionate access programs, phase 4 surveillance systems, patient registries, and patient support programs (PSPs) – an offering accompanying many specialty drugs – can all serve as rich data sources.

While PSPs exist primarily to guide patients through treatment, adherence and access, they can also track key clinical parameters – from liver enzymes to disease markers, joint erosion to tumour progression. As integrated support systems for patients with a common disease, PSPs also serve as ideal vehicles for collecting patient-reported outcome measures (PROM) – an increasingly recognized means of assessing treatment success – such as quality of life and patient satisfaction.[9]

This data engine can feed back to clinicians, allowing them to gather information on disease scores, adherence, and persistence, and use it to better meet their patients' needs. Pharmacists, who interact with both clinicians and patients, have a front-line perspective on patient behaviours. As the first point of contact for many patients, they can both document and influence adherence.

To earn stakeholders' trust, the data must be free of bias. We need the expertise to understand and "clean up" the biases that creep into real-world data, such as differences in record keeping or in physician prescribing patterns.[10] Patient-reported data, arguably most vulnerable to bias, may require third-party oversight and validation to pass muster. Above all, trust in the data will depend on establishing a dialogue in which all stakeholders have a say.

Patients, for their part, may fear that their medical data will get into the wrong hands, employers being a common concern. Fortunately, modern medical data collection has robust mechanisms for deidentifying patient data to avoid privacy leaks.

In an ideal world, stakeholders will not only collect the data but aggregate it into evidence and insights: how patients, prescribers, and payers are behaving, and why. These insights, in turn, will drive a fairer system for all players. Forward-thinking payers will have a basis for negotiating outcomes-based agreements (OBAs), which limit their financial responsibility for poorly performing drugs. Manufacturers will make better strategic decisions for existing and pipeline medications. And patients will get better care. For all this to happen, we need to decide who will take the initiative to obtain and interpret the data – and who will pay for it.

**The bottom line:** new specialty drugs are coming to market every day and patients are anxious to use them. Prescribers need to know which medications work best, and data from RCTs doesn't provide the full picture. Real-world data already exists, PSPs can capture a lot more, and policymakers recognize the need to take action. **So what are we waiting for?**

## Data Descriptors

**Data:** Quantitative information. Numbers. Trends. All the good stuff.

**Specialty pharmacy data:** All data collected at the dispensary and through patient touchpoints relevant to a specialty medication, ranging from objective and subjective clinical outcomes and disease progression to pricing, payers and demographics.

**Patient support program data:** Data on specialty indications/treatments generated within a PSP. PSPs provide a self-contained setting for the collection of disease-specific clinician- and patient-reported data, as well as operational program data.

**Real-world data:** Data on a medication's performance and cost-effectiveness in patients with real-life clinical profiles, as opposed to patients in clinical trials.

**Real-world evidence:** Evidence arising from the aggregation and interpretation of real-world data.

**Outcomes-based agreements:** Agreements between manufacturers and private/public payers in which the payer's responsibility to cover the medication is based on its performance in the real world.

**Predictive analytics:** Using data to create projections enabling the clinical and economic management of patients and their therapeutic journey. Management of adherence and disease progression is a current focus in the industry.

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# What We're Reading

We find that the following articles provide great insight into the specialty pharmaceuticals market. Follow us on **LinkedIn** where we're sharing our thoughts on these topics and many more.

- Rid yourself of dirty data
- IHE: Defining decision-grade real-world evidence and its role in the Canadian context
- Significant developments in Canadian drugs for rare disease policy
- How well do people do in the real world?
- Claudia Zanchetta: Diving into data at Cancer Care Ontario
- Podcast: RCT & RWE: Evolution, revolution or integration?
- Embracing big data to understand complex diseases
- Real-world patient- and caregiver-reported outcomes in advanced breast cancer

# Upcoming Issues

In upcoming issues of The *20Sense Report*, we'll take a deeper dive into:

- Specialty therapy innovations on the horizon
- Patient support programs: exploring opportunities for outcomes-based agreements
- The patient perspective on specialty pharmaceuticals

**Is there an issue you'd like us to address? Do you have a question you'd like us to answer?**

**We welcome your suggestions for topics you'd like *The 20Sense Report* to cover.**

Are you looking to make better sense of the specialty pharmaceuticals market?

**Contact us at:**
**info@20Sense.ca**
**www.20Sense.ca**
**LinkedIn**

20Sense helps pharmaceutical manufacturers and specialty service providers more effectively enter and compete in Canada's complex specialty pharmaceuticals market by optimizing data, insights and programs that deliver better outcomes for patients and value for payers.

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