THE 20SENSE REPORT

SPOTLIGHT ON THE CANADIAN SPECIALTY PHARMACEUTICAL MARKET

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20SENSE

Making sense of Canada's specialty pharmaceutical market

Data Surge Ahead

This much we know: healthcare data is on a roll. Now is our chance to ensure the growing data stays accurate, accessible, and actionable.

Big Data and Specialty Drugs: A High-Traffic Intersection

Which sector expanded its data capacity the most between 2016 and 2018? The answer, according to a Dell Technologies survey, is healthcare.[1] While the financial sector, telecommunications and IT boast a larger absolute quantity of data, healthcare data showed the highest *rate* of growth.

When you consider the volume of information processed in healthcare, this surge comes as no surprise. With new technology that makes it possible to translate physician notes, patient self-reports, and radiographic images into hard numbers, the byte count increases with each passing day.

In the high-stakes world of specialty drugs, this data boom represents an opportunity to capture real-world drug performance, use our funding dollars wisely, and ultimately match the right patients to the right drugs – but only if done right.

Getting creative

The quest for robust, actionable data has forced researchers and policymakers to grapple with difficult questions about data collection, privacy, and sharing. Adding further complexity to the picture, each province – and each institution, for that matter – has different regulations around data capture.

These hurdles have not prevented the sector from moving forward with new data initiatives, from pilot projects to publications, with each new success fostering the competence and confidence to reach still higher. No longer in its infancy, the data collection ecosystem has entered a more mature phase characterized by greater creativity and collaboration.

Alberta is leading the charge in data connectivity with the potential to generate real-world evidence (RWE). The province's Oncology Outcomes Initiative,[2] for example, serves as a model of data capture along the oncology product lifecycle. Manitoba has announced the upcoming launch of MindSet, a data platform that will link the province's disparate pockets of clinical data.[5] In a best-case scenario, academic, industry, and policy researchers could tap into MindSet's rich data vein for their studies. When will Ontario and Quebec, which account for over half of Canada's population, catch up? Encouraging efforts include the ConnectingOntario ClinicalViewer portal[3] and the Quebec Health Record,[4] which combine patient data from such sources as physician lab results, imaging reports and hospital visits, though these initiatives fall short of RWE generation. →

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South of the border, the health data economy has been busting out of its own sector. Earlier this year, in an unprecedented deal, hospitals granted access to patient information to Microsoft, IBM and Amazon.[6] The agreement, which respected patient privacy laws, brought to light hospitals' central role as data repositories and brokers. Similar deals may be coming to a hospital near you.

Looking still further ahead, a "data superhighway" that connects data from patient populations across our country no longer seems so difficult to imagine.

Real-world "magic"

A lot of the information being collected falls under the umbrella of real-world data (RWD). As defined by the FDA, RWD consists of "data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources," such as electronic health records, product and disease registries, and data generated by patients.[7]

So where does that leave data from randomized clinical trials (RCTs), the hallowed standard of medical evidence? As an all-star clinical trials group recently argued in a New England Journal of Medicine opinion piece, RWD can't replace the "magic" of randomization, and only RCTs can be trusted to capture smaller treatment effects.[8] Rather than supplanting RCTs, RWD can complement them by establishing the long-term efficacy and safety of a drug, including its effects in specific populations.[8]

No longer in its infancy, the data collection ecosystem has entered a more mature phase characterized by greater creativity and collaboration.

RWD can even do some of the grunt work for clinical trials. For example, electronic medical records can help identify large numbers of patients eligible for a trial, avoiding the inefficiency of enrolling small numbers of patients at multiple sites around the world.[8]

Juicing the data

Canada's specialty drug space draws on several sources of RWD, with patient support programs (PSPs) a notable addition over the past couple of years. Exemplifying this trend was a recent analysis using data from Taiho Canada's PSP for tifluridine/tipiracil, a treatment for metastatic colon cancer.[19] The researchers' conclusion: the rapid rate of enrolment in the PSP reflects a great clinical need, and prior chemotherapy is a factor in treatment discontinuation.[9]

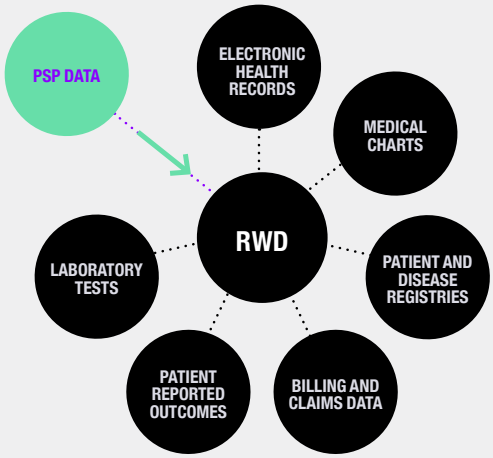
As noted earlier, efforts to link the disparate sources of RWD have been ramping up. For instance, some PSPs have been working to integrate laboratory services into their data sets, in view of improving workflow processes such as scheduling blood tests and communicating results to physicians.

At the same time, more and more data from patient registries and questionnaires has been filling gaps left by clinical research. The Canadian Kidney Cancer Information System helped researchers assess quality indicators for patients undergoing renal carcinoma surgery, while the Canadian Melanoma Research Network Patient Registry shed light on the real-world effects of new therapies for the disease.[10] In another instance, a survey completed by over 1,700 patients revealed a high level of satisfaction with the infliximab infusion experience, significantly exceeding patients' baseline expectations.[11]

The data is also becoming more structured. Until recently, data analytics has depended on the manual extraction of clinical variables from patient charts and research databases – a laborious and costly exercise.[9] Natural language processing (NLP) technology is changing all that. The technology can convert narrative chart reports into research-grade data sets. A Toronto-based data service provider is pairing this technology with AI methodologies to extract variables from clinical texts and turning them into row-column datasets.[12] Applying this process to the University Health Network in Toronto, the company successfully aggregated more than 1,400 records for lung cancer patients.[12] In a similar effort based in Alberta, NLP technology extracted machine-readable data – with 98.4% accuracy – from chart reports of multiple myeloma patients, thus helping to build a cost-effective infrastructure for advancing cancer care.[13]

All told, the specialty medicine sector is not only producing more data, but beginning to use it creatively to generate insights that drive access and clinical decisions. To serve our patients with complex medical needs, we need to do more of the same. A lot more.

THE RWD FEED IN CANADA



Q&A: At the Epicentre of Real-World Data: A Conversation with Winson Cheung

Dr. Cheung holds not one, but two positions with Alberta Health Services: Director, Real World Evidence at Cancer Control Alberta (CCA) and Principal Director of the Oncology Outcomes (O2) Initiative. As such, he spends his days analyzing real-world evidence (RWE) and advising stakeholders on its clinical value. A senior medical oncologist and Full Professor of Medicine at the University of Calgary, Dr. Cheung has published over 200 peer-reviewed manuscripts and also runs a weekly gastrointestinal cancer clinic. His academic research focuses on the interplay between patient, physician, and system-level factors that drive practice patterns in the real world.

Q: Tell us a little about your position as RWE director for CCA.

I feel very privileged to have this job, given that no other province in Canada has a similar leadership position. Alberta has a long tradition of generating real world data (RWD) and evidence, and this position takes it a step further.

Q: If you had to give an "elevator pitch" to convey the value of RWE, what might you say?

All healthcare stakeholders want the same outcome: a drug that helps people live longer and better. RWE helps us to achieve this objective. While clinical trials continue to have significant value, subjects enrolled in trials only represent 5 to 10% of the population. RWE broadens the evidence to include the general population. For example, RWE provides information on whether a drug is truly effective in a broad population and also offers insights into health-economic outcomes that can impact policy decisions. Stakeholders are increasingly recognizing RWE as an important complement to clinical trials and biomarker studies.

CLINICAL TRIALS	REAL WORLD EVIDENCE
Internal validity	External validity
Young and fitter patients	Older and frailer patients
Finite follow-up	Longitudinal follow-up
Tumor-specific	Tumor-agnostic
Singular primary endpoints	Multiple potential endpoints
Limited information on healthcare costs/use	Extensive information on healthcare costs/use
Granular data on selected patients	General data on unselected patients
Resource intensive	Relatively inexpensive

SOURCE: ADAPTED FROM DR. CHEUNG'S SLIDE PRESENTATION: "FROM REAL-WORLD DATA TO REAL-WORLD PATIENTS: THE O2 PROGRAM."

Q: It seems that RWE has become a "hot topic" in Canada. What might account for the trend?

The term "real world evidence" itself has given new life to the concept, which used to be known as health outcomes research. On a more fundamental level, Canada is a large, geographically dispersed country with many different populations. Stakeholders have come to realize that we need evidence on how drugs perform in these real-world populations – all with the aim of getting the right treatment to the right patient at the right time, and at the appropriate cost to payers.

Q: Who should be conducting RWE research?

While RWE is largely supported by industry at the current time, academic and hospital centres should also spearhead more RWE studies. As for payers, I see them in a more advisory capacity – letting researchers know what type of data they need – rather than conducting

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the studies themselves. Ideally, trained RWE specialists should be leading the design of RWE studies because they have a fuller grasp of the required methodology and potential sources of bias, and can thus generate more powerful evidence.

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Q: How might RWD/E help improve access to and use of specialty cancer drugs in Canada?

Many cancers are rare, making it difficult to find enough subjects to run randomized clinical trials. In such cases, RWE can bridge the data gap – for example, by serving as the historical or real-world comparator in a single-arm study. RWE can also answer specific questions such as: "Out of 100 people eligible and prepared to take drug X, how many did not receive it because of age or geographical barriers?" This helps to quantify the actual demand. By the same token, RWE can provide more accurate population-based projections of cost savings than traditional economic modelling that rests heavily on assumptions or extrapolations.

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Q: Can patient support programs (PSPs) be harnessed to generate RWE?

PSPs are a great opportunity for RWE in that they have a pre-existing data set that encompasses patients taking a particular drug. One potential downside is that the patient consent forms used in PSPs can sometimes make it hard for researchers to access the data, though this is evolving over time. When PSP developers consult me, I often suggest they create consent forms that enable us to interrogate the data in an anonymized and aggregate fashion. We have learned that the vast majority of patients support this idea.

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Q: Are there opportunities to share RWE across institutions and jurisdictions?

I am currently involved in an initiative to pool data across provincial borders, in hopes of generating more robust data that can be generalized across the country. Differences in data entry formats and privacy rules between provinces have created some unique challenges, but we are working through them with the right provincial leaders and the right software to facilitate data merging.

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Q: Are there any other ways to increase the efficiency and quality of RWE?

We have been collaborating with artificial intelligence companies to assist with data extraction. For example, one of our partner companies has the capacity to translate written physician reports (unstructured fields) into quantitative data (structured fields). Another company has the technology to extract quantitative data directly from images such CT scans. This saves us from needing to embark on the resource-intensive step of manually collecting and entering data from images and physician notes. While still in the early stages, these collaborations will help us to generate more comprehensive and higher quality data, which is very exciting.

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Q: You recently launched the Oncology Outcomes (O2) RWE program. Can you tell us more about what you hope to achieve with the program?

We created the program as a "one stop shop" for oncology RWE, to showcase Alberta's real-world data sources and to promote our strong analytics team to collaborators. All stakeholders are welcome to approach us. We conduct the studies in-house and, once we obtain all the necessary access and research ethics approvals, share aggregate level results with our collaborators.

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Q: What is your "blue sky" vision for RWE in the future?

At present, we have a tendency to look at RWE *after* a treatment has already reached the market and patients are already using the drug. We have opportunities to use RWE more creatively. We could generate evidence a lot earlier, perhaps even in parallel with clinical trials. RWE could even help guide clinical trial design and inform the commercial potential of a drug. In short, I believe that RWE has tremendous value across the entire lifecycle of a drug – before, during, and after clinical trials.

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Q: What might you like to convey to industry with respect to RWE?

The key message is to plan ahead. It is not unusual for me to get requests for RWE a few weeks before it is needed, which is not realistic. High-quality RWE takes time. I tell companies to give me at least a year's notice and to aim for scientific publication, as unpublished RWE is not perceived as robust. If payers and regulators are to take RWE seriously, we need to apply the same rigour to the process as we do to clinical trials. We must also be prepared for both positive and negative results, just as with clinical trials. All research involves risks, but the rewards can be significant if we end up with data that has tangible meaning for real patients in the real world.

Paving the way – RWE from the Taiho Patient Support Program

Can real-world data from PSPs influence practice and policy decisions? Dr. Cheung put this idea to the test in a 2018 study. In the interest of assessing unmet needs in the treatment of metastatic colon cancer, Dr. Cheung and his research team analyzed real-world treatment patterns for trifluridine/tipiracil (Lonsurf), a refractory colon cancer treatment that many patients were seeking. Specifically, the team looked at RWD from the Lonsurf PSP run by Taiho Pharma Canada and from Health Canada's special access program for the unfunded treatment.

The analysis included over 700 Canadian patients, most of them enrolled in the PSP during a 7-month period in 2018. At the end of the study period, 32.8% remained on treatment. Among those who stopped, reasons included disease progression (51.9%), decision of the treating physician (19.3%), and death (13%). Notably, only 4.4% discontinued treatment due to toxicity and an additional 4.4% withdrew their consent. "The fact that many people remained on treatment for several months told us that the drug had value beyond a clinical trial population," Dr. Cheung explains.

And did the analysis move the funding needle? "While this particular drug remains unfunded [at time of writing] in most of Canada, this paper has caught the attention of a number of other companies, many of them interested in generating similar data that could inform funding decisions," says Dr. Cheung, who expects to see "more and more PSPs being harnessed to produce actionable RWE."

The study was published in Current Oncology in 2019. https://bit.ly/34G6jQb

Around the Bend: What's Coming and Why it Matters

Over the coming months and years, the data surge will continue unabated – not only in volume, but in quality and breadth. "Data for the sake of data" will no longer cut it: the information collected will need to serve an increasingly clear purpose. Here's what to watch for.

Straight from the Source

Data generated from patients themselves, known as patient-reported outcome measurements (PROMs), is on an upswing. Take CAPTURE ALS, a pan-Canadian database of amyotrophic lateral sclerosis (ALS) patients in current development. The data collected will range from biologic samples to PROMs, giving researchers a clearer path to personalized treatments.[14] And who better to validate PROMS than patients themselves? That's what's happening in an agreement between the Coalition Priorité Cancer du Québec (a patient group) and Value-Based Health Canada, which has patients validating PROMs for breast, lung, and colorectal cancer to help identify gaps and inefficiencies along the care continuum.[15]

High Performance

How can our healthcare system bear the load of specialty drugs without compromising patient health? Stakeholders are turning to RWD to solve this puzzle. By shining a lens on a drug's performance in the real world, RWD can help funders make decisions that support best care. Indeed, a 2019 report from the Advisory Council on the Implementation of National Pharmacare recommends using RWD to underpin performance-based funding agreements for rare disease drugs.[16] The more data collected over the long term, the greater its power to help patients access effective drugs with the minimum delay.

Fair Play

Nobody wants a repeat of the 2018 Facebook scandal, in which millions of Facebook users saw their personal data harvested without their consent.[17] As big data gets even bigger, concerns about privacy will become still more pressing. Prepare yourselves for a lot of conversations about the social implications of collecting and using patient data. Questions to hash out include: Why exactly is the data being collected? Does it infringe on basic rights? Does it favour certain patient groups over others? A case in point: Winterlight Labs, a Toronto start-up using speech recognition technology to identify neurological diseases such as Alzheimer's, soon discovered that the technology only worked for Canadians with a particular dialect.[18] A priori discussions between the data scientists, doctors and patients could have prevented this "oops."

Above all, all parties need to agree on how the data will be used. Consensus takes time, but the gears are in motion.

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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.

- Real-World Data Can't Replace the 'Magic' of Randomized Trials
- The new project integrating Manitoba's clinical health data into one platform
- Hospitals Give Tech Giants Access to Detailed Medical Records
- The public needs to know why health data are used without consent
- AI & Real-World Data: A Perfect Union
- If AI is going to be the world's doctor, it needs better textbooks

Upcoming Issues

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

- The impact of COVID-19 on the Canadian specialty pharmaceutical industry
- Exploring opportunities for outcomes-based agreements with specialty pharmaceuticals
- Patient perspectives 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.

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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