THE 20SENSE REPORT

SPOTLIGHT ON THE CANADIAN SPECIALTY PHARMACEUTICAL MARKET

OCTOBER 2019 ISSUE 10

20 SENSE

Making sense of Canada's specialty pharmaceutical market

At the Heart of It All: The Specialty Pharma Patient

Complex Patients in a Complex World

How much difference a single year can make. In 2019 alone, policy changes have torn through the fabric of specialty medicine, keeping stakeholders on their toes all year long.

For starters, the federal government's 2019 budget included steps toward a national Pharmacare program, including a national drug agency, a national formulary of evidenced-based drugs, and a strategy for high-cost drugs for rare diseases.[1] Along similar lines, the Patented Medicine Prices Review Board (PMPRB) announced a series of reforms to make drug pricing more transparent, enabling the agency to set price ceilings and make specialty medicines more affordable to Canadians.[2]

As expected, not all manufacturers are on board with these changes. Indeed, a group of manufacturers plans to take the federal government to court over the PMPRB reforms, maintaining that Ottawa lacks the constitutional authority to set price ceilings.[3]

Changes have also rattled the world of biologic medications, spearheaded by the BC government's expanded use of biosimilars – functionally equivalent versions of originator biologics that cost up to 50 percent less – within its provincial formulary.[4]

These upheavals have also nudged patients into new territory. As they brave increasingly complex treatment regimens and stick-handle around access hurdles, they are finding their voices and seizing opportunities to speak up. So how are they handling it all? How much sway do they have in their own treatment? Are their voices landing in the right ears?

As the science and business of specialty medicine charges ahead, stakeholders need to keep an eye on the patient perspective – to shine a light on what patients want, lack and need, and what drives them to push forward.

Engaged to the max

The term "patient engagement" may have buzzword status, but the phenomenon is real. Today's patients not only have professional-level knowledge about their disease, but also keep up with policy changes – and speak up when they disagree.

When the PMPRB announced its pricing reforms, the patient-led Canadian Organization for Rare Disorders (CORD) responded with a flurry of news releases and marketing campaigns to communicate their dismay. They didn't mince words, either. "Either the federal government can't understand the disastrous impact on patients with high needs or they simply don't care," said CORD president Durhane Wong-Rieger in a press release.[6] Concerned the price drops required by the new regulations would dissuade pharma companies from playing in the Canadian market, Wong-Rieger predicted the changes would "compound our existing drug shortages" – and more importantly, hurt patients.[6]

In response to the outcry, the PMPRB assured stakeholders that it will be "more forgiving in the ceilings that we apply to [rare disease] drugs, because we want to be consistent with the government's broader commitment to trying to facilitate access."[7] While the chips have yet to fall, the ongoing dialogue exemplifies patients' burgeoning role in shaping policy.

The sea change goes both ways: more patients want to speak up, and more policymakers want to hear from them. →

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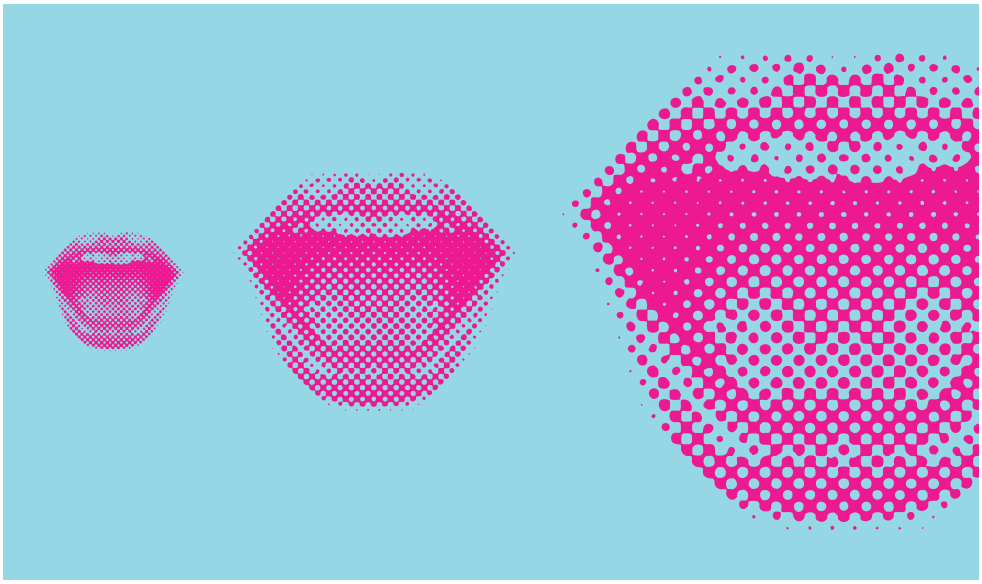

Following a call for nominations, the Committee for the Assessment of Devices and Technologies in Health (CADTH) assembled a new Patient and Community Advisory Committee composed of 12 patients and caregivers of diverse backgrounds.[8] According to a CADTH press release, the committee will "advise CADTH on all areas of the agency's work" – an ambitious vision that, if all goes well, will help steer CADTH toward more equitable and appropriate recommendations.[8]

A full-time job

With all the opportunities to weigh in on research, access and policy, people with complex diseases can make a career of being a patient – and some of them do just that. Take Lisa Machado, for example. About a decade ago, Machado developed Chronic Myelogenous Leukemia (CML), a type of leukemia diagnosed in 500 Canadians every year.[9] Finding no group dedicated to the disease, Machado created one of her own. Called the Canadian CML Network, the group is working on putting together a Task Force on Rare Cancers, working on a peer-to-peer training program, and joining forces with global oncology organizations on advocacy initiatives.[10]

Machado herself has launched Canada's sole educational conference on CML, created and deployed a Novartis Oncology-backed social media campaign on leukemia, and written an award-winning CML educational resource. Along the way, she has also become a sought-after public speaker, invited by such leading broadcasters as CBC and CTV to share her perspective as a patient.[10]

Machado's passion comes through in every word she says and writes about the patient experience. From an article titled Lives Depend on Patient Engagement:[11] "When I laud an institution for attempting patient engagement, it's because I have seen the other side of the bed and know without a doubt that involving patients is critical to a system that works and achieves better outcomes."

As if advocacy leader, public speaker, and medical journalist weren't enough, some patients and their families reach into the science of medicine itself. After learning there was no effective treatment for his young daughter's ultra-rare neurologic disease, alternating hemiplegia of childhood, a US real estate developer called Simon Frost – with no background in science – set about developing a gene therapy for the disorder.[12] Now an inventor with international patent applications, Frost has also become a project manager who coordinates the research.[12] As Frost's trajectory exemplifies, today's patients have limitless means to make change – and many devote their lives to the effort.

It gets complicated

Hope is what drives patients to get involved, and new specialty medicines offer hope to the most treatment-resistant patients. But this promise comes with a high level of complexity, making it critical to set things up so patients can benefit from the innovations without undue strain on their time, energy, and mental reserves.

Complexity may crop up before treatment even begins. Eligibility for the recently-approved anti-cancer drug Vitrakvi, for instance, rests on confirmation of a very specific gene mutation, requiring patients to undergo genetic testing to find out if they qualify.[13] With similar treatments multiplying in the pipeline, hospitals and clinics will need to expand their genetic testing infrastructure to meet the increasing demand.

Eligibility hardly guarantees access, of course. In a striking illustration of the difference, André and Joshua Larocque, two young brothers living with cystic fibrosis (CF) in Tottenham, Ont., have found themselves on very different treatment paths. André, 8, was selected to enter a clinical trial of a promising new CF medication called Symdeko. Health Canada has already approved a similar drug, Orkambi, but the province won't pay the $250,000 annual tab for six-year-old Joshua to take it, based on a negative recommendation from CADTH.[14] The decision doesn't sit well with the boys' parents, who have been lobbying the province to fund the medication.[15] For better or worse, the realities of specialty medicine have thrust the Larocques into challenging new roles.

As specialty medicines keep tumbling into the market and policies continue to shape-shift, patients will raise their voices still further, blurring the lines between healthcare user and advisor. As Machado put it in a recent online article, "Patients and caregivers are experts in their field, like any consultant. Why are we still debating whether or not to compensate this expertise?"[16] One thing we know for sure: the patient voice is here to stay, and stakeholders need to listen more closely than ever.

Defined By Attitude, Not ALS

For a loud and clear patient voice, we need look no further than Adam Welburn-Ross. A shining example of the expanded patient role, Adam has explored every corner of specialty medicine.

Innovative drug treatment backed by a patient support program? *Check.*

Clinical trial? *Check.*

Health outcomes reported to a registry? *Check.*

Patient advocacy? *Quadruple check.*

As passionate about life as he is about research and patient care, he wants no part of the victim role. So how does this super-patient maintain such energy and grace? What can he teach us about the specialty drug patient of the future? We spoke to Adam to find out.

If you expect Adam Welburn-Ross to act like a sick person, you're in for a disappointment. Adam, 44, doesn't roll that way. He's too busy living his life. And writing letters to Members of Parliament. And joking with his doctors. And laughing – a lot.

Not that Adam has his head in the sand. A father of two young-adult children, he knows perfectly well that the disease he lives with, amyotrophic lateral sclerosis (ALS), gradually robs people of the ability to walk, talk, eat, and eventually breathe.[1] He knows that ALS leads to changes in thinking and personality in up to half of affected people.[2] He knows that the average ALS patient lives just two to five years after diagnosis.[2] He knows all this, and yet he has no time for despair. "I focus on living in the moment," he says, "and at the moment things are great."

Adam's optimism has strong roots in his life. During his 10 years as a hockey coach, his unrelentingly positive attitude earned him the nickname "coach sunshine." Now that the mountain of ALS looms ahead of him, "I have to practise what I've been telling the players all along – to stay determined and never quit. It's been a really important factor in my attitude since the diagnosis."

The long road

Diagnosed for the first time in 1869, ALS is a neurologic disorder that blocks communication between the brain and the muscles, leading to progressive paralysis. Adam received his own diagnosis in mid-2018, after a year and a half of undergoing "inconclusive" investigations and waiting to see specialists. And waiting. And waiting.

That's when his self-advocacy instincts kicked in. He asked a relative who worked in health care for help navigating the system, which led to a referral to a clinic that conducted "every test imaginable," which led to a tentative diagnosis of ALS. He eventually found his way to Dr. John Turnbull, who heads the ALS clinic at the Hamilton Health Sciences Centre and confirmed the diagnosis. "If I were passive, there's a good chance I still wouldn't know what disease I have," Adam says. "And that's not right. We need to shorten the time to diagnosis and treatment."

Adam clicked with Dr. Turnbull from the start. "He has a patient focus, rather than a disease focus," he says, explaining that "he doesn't just ask me how my leg is doing, he asks me how I'm doing as a person. He also encourages me to bring caregivers to my appointments, and checks in with them as well."

Under Dr. Turnbull's supervision, Adam began treatment with Rilutek, an oral medication that slows the progression of ALS symptoms,[19] and joined the →

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medication's patient support program. His treatment journey took a twist in the fall of 2018, when Health Canada approved a new ALS medication called Radicava. With his interest in scientific innovation and appetite for new experiences, one might think Adam would be the first in line for the new drug. However, the drug's on-off IV dosing gave him pause – a reservation shared by Dr. Turnbull, who wrote a peer-reviewed journal article questioning the overall value of the medication[20] – and he stepped away from the opportunity.

A champion of patient rights, Adam views fighting for treatment and stepping back from treatment as two sides of the same coin. "We have the right to an opinion about our own treatment," he notes, reflecting the rising tide of patient agency in specialty medicine. "This includes the right to say no."

The super-patient

Intent on leaving a legacy to future ALS patients, Adam embodies everything that today's specialty medicine patient can be and do. On the advocacy front, he collaborates tightly with ALS Canada to prod governments into action – specifically, to make ALS drugs more accessible and affordable, to improve home and community care for ALS patients, and to allocate more funds to ALS research. Some of the items on his wish list are quite simple – "like a manual for ALS patients and caregivers on how to navigate various health care systems and a contact list for related resources."

He has also enrolled in a clinical trial of levosimendan, an oral medication shown in previous studies to help the muscles contract more easily.[21] The trial will evaluate the effect of the medication breathing function[21] – an exciting hypothesis, but not the reason Adam signed up. "To tell you the truth, I didn't care which trial I got into," he says with a laugh. "I just wanted to help move science and ALS treatment forward." It helps that the medication hasn't given him any side effects. "If it made me sick, I probably wouldn't be quite as enthusiastic." A further bonus: if the results pan out, he will receive the drug at no cost for the rest of his life.

All told, Adam highly recommends the clinical trial experience. "As soon as this one is over, I plan to join another one," he says. In the meantime, he has been tracking his symptoms and day-to-day activities and sending the information to a US-based real-world data collection group called the ALS Therapy Development Institute. "It's an honour to share my health data in the name of progress," is how he sees it.

In May 2019, when Adam still had full mobility, he participated in the Walk to End ALS, the largest volunteer-led fundraiser for Canadian ALS societies.[22]

"We're after the brass ring – an actual cure – rather than improvement of symptoms," he says. A hundred-and-fifty years after the first diagnosis, ALS remains a terminal disease – and Adam is not OK with that. In his view, "research aimed at a cure aligns more closely with Canadian values," and he would like to see "the government agenda reflect the fabric of our country."

A new normal

More than a year after his diagnosis, Adam can no longer walk unaided. After experiencing one too many instances of foot drop, which prevents the foot from recoiling and throws off gait, he began using a walker, and he now gets around in a wheelchair. Accepting that he has entered a new, less independent phase of his life, Adam relies on his partner, Danielle, to help him with day-to-day activities. He can still brush his teeth, but "Danielle makes it easier by putting my toothbrush and glass of water within reach," he says. If he needs a drink of water or juice, she opens the bottle for him. If he has to leave the house, she loads his wheelchair into the car.

Adam takes none of it for granted. "I sometimes think it's even harder for caregivers than for patients," he says. "In a better world, the system would reward caregivers for their Herculean efforts." For the time being, he has been able to access up to 21 hours of care per week. The rest falls to Danielle, leaving her with a double-shift of full-time work and after-hours caregiving. "She's not only risen to the challenge, but she's kept her spirits high," Adam says, conceding that "my crazy positivity probably rubbed off on her." His attitude has also inspired his mother, Gail, to follow his lead. "I told her that I'm alive right now, so let's enjoy our time together, and that's what we've been doing. When you get right down to it, the only thing we ever have is today."

"We have the right to an opinion about our own treatment," he notes, reflecting the rising tide of patient agency in specialty medicine. "This includes the right to say no."

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Champion of Cancer Patients: Interview with Alan Birch

Alan Birch's job description – drug access facilitator – has grown out of the increasing challenge of accessing innovative medications in Canada. As part of the cancer care program in a large Toronto hospital, Alan spends his days helping patients obtain cancer medications as quickly and with as much financial support as possible. A pharmacy technician by training, Birch brings a scientific understanding of specialty medications to his work. Currently a director at the Oncology Drug Access Navigators of Ontario (ODANO), Alan helps raise awareness of his profession and teaches less experienced colleagues to navigate the access landscape. In this interview, Birch shares his unique front-liner's perspective on the hurdles faced by cancer patients requiring specialty medicines.

How would you describe your role?

I help expedite access to complex oncology medications. When a doctor prescribes such a medication, I launch into "investigation mode" to identify and clear away barriers to access and reimbursement. While many patients get automatic coverage, others fall through the cracks because of their age, their insurance status, or a number of other circumstances. In such cases I help connect these patients with various reimbursement channels, compassionate care programs, or special access programs for drugs awaiting approval.

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Q: Why are access navigators more prominent in oncology than in other specialties?

There are over 200 different types of cancer, with new drugs coming down the chute all the time. Our current reimbursement system was not designed to handle so many specialty drugs at such high prices, and nobody is lining up to pay the bills. Patients can't be expected to know about all the alternative sources of financial support, which is where the access navigator comes in. Patients routinely contact me with questions like "I spent $1,000 on a drug, now what?" or "I'm having trouble with my Trillium application," so the access navigator role does seem to fill a need.

Q: What is the "typical" experience for patients prescribed a complex oncology therapy?

The patient experience is constantly changing, and always in the direction of complexity. It used to be that a patient came into the clinic, received an infusion of chemotherapy, and left. As we move toward targeted therapies, meaning treatments tied to specific gene mutations, patients may need to get their tumors genetically tested to find out if they're eligible for a specific treatment. The province covers only some of these genetic tests – generally the more basic ones. Patients are left wondering whether to pay out-of-pocket for a more sophisticated test that may or may not point them toward the best treatment choice.

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Q: What are some of the key challenges facing oncology patients today?

If there is any common theme, it would be time. A delay here, a delay there, and the patient ends up missing the window of opportunity for best treatment. The delays are frustrating to watch and still more

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frustrating to experience. It can get to the point that the doctor gives up and switches the patient to the standard of care. Not that the standard of care is a bad thing, but naturally the patient wants access to the most promising options. It also seems unfair that some patients can access these new medications without any problem, perhaps because of their age or insurance plan, while others, through no fault of their own, have to wait and wait.

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Q: Are governments doing anything to simplify access for patients?

We're in a transition period and changes are coming. For example, the Ontario government has created the SADIE [Special Authorization Digital Information Exchange] portal, which reportedly makes it easier to submit requests for exceptional access to drugs. The portal will be available to drug access navigators next year. A genetic test called OCTANE, involving about 50 oncology genes, is currently running through clinical trials at major Ontario cancer clinics. We hope the provinces will eventually fund the test, but the possibility is still years away.

As we all know, the discussion around national pharmacare keeps heating up – a double-edged sword, in my view. I appreciate that national pharmacare may help level the playing field, but if you eliminate private insurance plans and don't speed up the timeline to get drugs on public formularies, you end up cutting off access. And that's not OK for cancer patients.

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Q: Where does the patient support program (PSP) fit into all this?

I see PSPs as another double-edged sword: on the one hand, they are critical to oncology care as they help patients get drug more quickly through bridging programs. On the other hand, PSPs have multiplied so quickly in oncology that cancer clinics can hardly keep up. Each PSP does things a little differently – for example, some have built-in compassionate access, while others only provide financial assistance – which makes for a highly fragmented care pathway.

Q: What advice might you give manufacturers of complex oncology therapies?

Speak to the clinics before designing your PSPs! Speak to the nurses, administrators, and access navigators. We know how things flow in a cancer clinic and the pain points along the road. Come in with an open mind and ask us questions. Build flexibility into your programs so that clinics can opt in and out of various services. When time is of the essence, consider providing product to oncology centres in advance, so they'll have it on hand to support timely bridging.

Perhaps most important of all, programs need to be more nimble. A delay of several days while waiting for a patient to sign off on a PSP enrolment is unacceptable. If the patient doesn't respond, you don't just file them as "unable to reach," you call again and again.

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Q: Overall, how can we do better for oncology patients?

Oncology patients have so much on their minds – the shock of receiving the diagnosis, telling their families about it, taking time off work, and dealing with medical appointments and lab tests – and don't have the time or energy to worry about access. It's up to us health professionals and policymakers to take the lead – to clear the path toward fair and timely access so cancer patients can concentrate on their treatment and their health. As a front-liner in the system, I have the privilege of working not only with patients but with other health professionals who share my passion for positive change, so I know we can do it.

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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.

- Power to the patients
- Filling the medication gap
- 'Canadians are clear': Latest CMA polls show we want to manage our health online
- When the illness is a mystery, patients turn to these detectives
- New committee brings diverse patient, family, and caregiver voices to CADTH
- Down the rabbit-hole: Being (a) patient in a fragmented system
- His daughter Annabel has a rare disorder. He's developing a novel gene therapy.
- 'Nobody can afford this': Family left helpless after Health Canada approves official version of prescription eye drops

Upcoming Issues

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

- Exploring opportunities for outcomes-based agreements with specialty pharmaceuticals
- Genetic-testing, specialty therapies and the growing complexity of specialty treatment
- Patient support programs: a ready infrastructure for health-outcomes data collection?

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?

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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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