20 SENSE

# THE 20SENSE REPORT

Spotlight on the Canadian Specialty Pharmaceutical Market

July 2022
*Issue 21*

# Innovation in Motion

The specialty pharmaceutical pipeline continues to outdo itself with disruptive new treatments

**The big, bold world of specialty drug development**

**Canadian stakeholders lay down the infrastructure to support innovation**

**IQVIA's Mark Omoto outlines a bright future for specialty medicines**

# By the *Numbers*

People may still be getting sick, but specialty drug development has never been healthier. Drugs for diseases previously considered untreatable are flowing through the pipeline, poised to change lives. Stakeholders had better hold on tight: this brave new world will only get bigger and bolder.

## BIRD'S-EYE VIEW

**$1.8 TRILLION**

Expected size of the global medicine market in 2026, after a 3-6% annual growth rate between now and then.[1]

**44%**

Dollar share of the Canadian drug market allocated to specialty medicines, up from 29% in 2014.[2] The figure is set to increase still further.

**ALMOST 8,500**

Number of new medicines at various stages of evaluation in 2021, compared to just under 7,000 the previous year.[3]

**5,500**

New clinical trial starts reported in 2021, up by 14% from 2020 and by 19% from 2019.[4]

## ONCOLOGY ON TOP

**37%**

Share of the pipeline devoted to oncology, the leading area of R&D.[4]

**> 40%**

Rare-cancer share of the oncology pipeline.[4] Most drugs being developed for rare cancers are precision medicines that target specific tumour types.

**100**

Number of new oncology treatments projected between 2021 and 2026, boosting the total 2026 oncology drug market to more than $300 billion.[1]

**17**

New cancer drugs in Canada in the first half of 2021, compared to 15 for all of 2020.[5,6]

## OTHER THRIVING AREAS

**30**

Cell and gene therapies launched globally to date,[1] of which 23 have received FDA approval as of early March 2022.[7] These therapies are used across a range of therapeutic areas including oncology.

**55-65**

Cell and gene therapies expected to launch by 2026.[1] The pipeline is overflowing, with over 3,000 cell, gene and RNA therapies in development.[7]

**39%**

Expected growth of the immunology drug market between 2021 and 2026, driven by new products for psoriasis, atopic dermatitis, and asthma.[1]

**4**

Disease areas – migraines, Alzheimer's, Parkinson's, and rare neurologic conditions – that are expected to drive the rapid acceleration of the neurology market.[1]

## MEETING A NEED

**> 40%**

Proportion of Canadians expected to get a cancer diagnosis at some point in life. About 25% of Canadians will die of the disease, making cancer the leading cause of death in the country.[8]

**> 500,000**

Number of Canadians living with dementia, of which Alzheimer's disease is the most common form. The figure is expected to rise to 912,000 by 2030.[9]

**72%**

Proportion of drugs that received an expedited review by the FDA in 2021, attesting to the strong public need.[4] Of these, about a quarter received an accelerated approval or emergency use authorization.[4]

**64%**

Percentage of 2021 new active substance launches in oncology that were approved through an accelerated process.[10]

# *Innovation* In Specialty Medicine: A Pipeline To Watch Closely





**Quick, a plumber! The specialty medicine pipeline is bursting.** Medicines that could have been lifted from sci-fi novels are on their way to clinics, hospitals, and pharmacies throughout Canada. While R&D is flourishing in many therapeutic areas, data analysts have teased out oncology, neurology, and immunology as key drivers of innovation.[11]

All this is both exciting and daunting. There's nothing simple or economical about these new medications, which require a complex infrastructure to support them. For example, patients may need sophisticated tests, including genetic analyses, to find out if a treatment is suitable for them. Once on the drug, they may need a battery of tests to ensure the drug remains a good fit.

How will the Canadian infrastructure handle this brave new pipeline? All signs suggest that stakeholders are ready to ride the wave of innovation and rise to the challenges along the way.

## EXCITING TREATMENT AREAS TO WATCH

### Oncology

Rare cancers, advanced cancers, cancers previously considered untreatable... scientists are working on treatments for them all. Indeed, cancer alone accounts for over a third (37%) of the specialty drug pipeline.[4] That's 2,226 cancer products in current development.[4] Canada alone saw 17 new cancer drugs approved in the first half of 2021, a significant jump from the 15 that received approval in all of the previous year.[5] Globally, the 5 years leading up to 2026 will usher 100 new oncology treatments into the toolbox, pushing the oncology drug market over the $300-billion mark.[1]

Even less prominent areas of oncology, such as kidney cancer, are welcoming new treatments every year. At the same time, researchers are moving beyond organ-based categories like "kidney cancer" or "lung cancer," having discovered that the genomic characteristics of a tumour matter more than its location in the body. This insight has spurred the development of tumour-agnostic therapies, which target a tumour's genomic profile, rather than its morphology or site.

Traditionally, oncology clinical trials have focused on patients with the most urgent needs, namely those with advanced cancers. This is changing: the past decade has seen a nearly two-fold increase in early cancer and cancer vaccine trials, with almost 200 trial starts in 2021 – 11% of the total for oncology.[10]

Innovation in advanced cancer treatment still dominates the scene, though, with superstars like HER2-positive breast cancer drug Enhertu grabbing headlines. Results of pivotal Enhertu trials showed a 49% reduction in disease progression as well as a 7-month survival advantage over chemotherapy in patients with hormone-receptor-positive and HER2-low tumours,[12] which experts viewed as "stunningly successful."[13] In an interview for the *New York Times*, Dr. Halle Moore, director of breast medical oncology at the Cleveland Clinic, said "it is unheard of" for a metastatic breast cancer drug to improve survival to this extent, adding that "few people expected the drug to work [in HER2-low tumours] because other drugs for such cancers had failed."[13]

**Oncology medication to watch**

| | |
|---|---|
| **Medication** | Enhertu (trastuzumab deruxtecan) |
| **Company (in Canada)** | AstraZeneca |
| **Indication** | HER2-positive breast cancer |
| **Distinctive feature** | The drug seeps through the cell membrane, and can then move into nearby cancer cells and kills them.[14] |
| **Newsworthy data** | In a pivotal Phase 3 trial of patients with HER2-low tumours, patients in the treatment arm survived for 23.9 months, compared to 16.8 months for patients who received standard chemotherapy.[13] |
| **Status in Canada** | Approved in Canada for adult patients with unresectable or metastatic HER2-positive breast cancer previously treated with trastuzumab emtansine[15]<br><br>Currently going through HTA review, with draft recommendation expected in September 2022[16] |
| **Other** | A patient support program (PSP) is in place in Canada.[17] |

### Cell and gene therapy

Cell and gene therapies have brought the future to our doorstep. These advanced technologies target disease at its most basic source: defective genetic material. This means we can now think of curing, rather than merely treating, chronic diseases.

While the strategy of transferring healthy cells to a patient has a long history – the first bone marrow transplant occurred more than 50 years ago – recent advances have made it possible to reprogram cells to help the body repair itself.[18] Gene therapies operate on a similar principle, though the transferred material consists simply of repaired genes, rather than whole cells.[18] And some treatments, such as

stem cell transplantation following genetic modification, combine both approaches.[19]

Over the past two decades, the discovery of several genes responsible for disease-causing mutations has injected new life into the cell and gene therapy market. As of May 2021, the pipeline had 1,745 therapies at various stages of development.[20]

**TERMS OF THE TRADE:**
**THE LEXICON OF CELL AND GENE THERAPY**

**Gene therapy**

A technique for the treatment of genetic disease, involving replacement of a missing or defective gene with a healthy gene.[3]

**Cell therapy**

Transfer of live cells to a patient to help cure or treat a disease. The cells may come from the patient (autologous cells) or a donor (allogeneic cells).[19]

**CAR-T therapy**

A type of cell therapy that uses modified T cells to fight cancer.[21] A patient's T cells are collected and modified to produce chimeric antigen receptors (CARs) on their surface. These new receptors latch on to antigens on tumour cells and kill them.

**Gene editing**

Technologies such as CRISPR and TALON, which modify (edit) DNA.[22] They can be used to induce T-cells to produce the CAR receptors needed for CAR-T therapy.

**RNA therapy**

While gene therapies make changes to DNA, RNA therapy alters the RNA in people with genetic diseases.[23] The corrected RNA is then used to create the protein that was missing or defective.

Oncology remains the most active locus of cell and gene therapy research, with more than 1,300 candidate products in development,[20] but these therapies also find niches in such areas as cardiovascular disease, respiratory disease, dermatology, neurology, and rare diseases, to name a few.[20]

One of the most prominent success stories in cell therapy, called CAR-T, involves manipulating cancer patients' immune cells to turn them into cancer-killing cells. Especially effective for lymphomas and leukemias, this disruptive technology offers lasting remission to some cancer patients previously considered terminal – patients such as Emily Whitehead, one of CAR-T's first success stories. Diagnosed with leukemia when she was just 5 years old, Emily, who lives in Pennsylvania, relapsed twice and her disease became resistant to treatment. Her only hope was CAR-T, and it didn't disappoint: Now a vibrant teenager, Emily has been cancer free for over 10 years since her treatment, which made headlines globally.[24]

Canada has also been ushering in new CAR-T treatments – a notable example being Abecma, which received Health Canada approval in May 2021.[3] The first CAR-T therapy approved for multiple myeloma (MM), Abecma modifies a patient's blood cells to attack MM cells that express a specific antigen called BCMA, as do nearly all MM cells.[25] While CADTH gave Abecma a "do not reimburse" recommendation in February 2022, citing insufficient evidence about duration of response and survival as a rationale,[26] the medication finds itself on many "to watch" lists, including Canada's own Meds Entry Watch.[3]

"One and done" treatments such as Abecma disrupt traditional care pathways and pharmaceutical business models. Scaling such treatments to generate sustainable value in Canada will require some new thinking.

**Cell therapy medication to watch**

| | |
|---|---|
| **Medication** | Abecma (idecabtagene vicleucel) |
| **Company (in Canada)** | Bristol Myers Squibb |
| **Indication** | Multiple myeloma |
| **Distinctive data** | The first CAR-T cell therapy for multiple myeloma[27] |
| **Newsworthy feature** | In a trial of 100 patients who had received at least 3 other treatments, 72% of subjects showed a response and 29% showed a complete response.[28] |
| **Status in Canada** | Approved in Canada for adult patients with multiple myeloma who have received at least 3 prior therapies for the disease[26] |
| **Other** | Abecma costs approximately $545,000 per infusion.[26] |

In the meantime, Canadian researchers have been conducting their own cell and gene therapy R&D. An exciting case in point: Massimiliano Paganelli, head of the liver tissue engineering and cell therapy lab at Montreal's Sainte-Justine children's hospital, and his stem-cell biologist wife, Claudia Raggi, have developed a cell therapy for children with liver disease.[29] They launched a biotech company, Morphocell Technologies, to test and commercialize the product, which they hope to deliver to patients within 2 years.[29]

**Neurology**

We're playing with a double-edged sword: life expectancy has shot up over the past century, but advanced age – one of the major risk factors for neurodegenerative disorders – compounds the opportunities to develop such diseases as





Alzheimer's, Parkinson's, stroke, and amyotrophic lateral sclerosis (ALS).[30] Isabelle Aubert, a senior scientist at Sunnybrook Research Institute and Canada research chair in brain repair and regeneration, foresees a "tsunami of aging disorders" in the years to come.[30]

The most common of these disorders, Alzheimer's, affects one in 9 people over age 65 and one in 3 over 85.[31] More than 500,000 people currently live with Alzheimer's and other forms of dementia in Canada, and if current trends continue, dementia will affect over 900,000 Canadians by 2030.[9] With such figures looming ahead, it's hardly surprising that scientists have doubled down on the search for effective treatments. The area has proven itself extraordinarily difficult to master, however, and after over 300 failed trials across the industry,[32] many companies have pulled out.

The meteoric rise and fall of the drug Aduhelm in the U.S. exemplifies the turbulence of Alzheimer's research. After a contentious accelerated FDA approval in June 2021, based on the surrogate endpoint of reduction in amyloid plaque,[33] the product languished in the rollout phase. Unconvinced by the data, many physicians and major medical institutions refused to support it.[34] Following suit, some insurers said they wouldn't cover it. In reaction to these developments, manufacturer Biogen made the decision to "substantially eliminate" its marketing budget for Aduhelm.[35]

Even so, things are looking up for Alzheimer's treatments, largely because clinicians can now diagnose the condition at a molecular level. "For so many years, we could not diagnose it until someone died," notes Sara Manning Peskin, assistant professor of clinical neurology at the University of Pennsylvania and author of the book *A Molecule Away From Madness*. "Now we have the tools that we can pick up in living people, [namely] the proteins linked to Alzheimer's," she says.[36] This technological advance has "opened up huge doors for clinical trial drugs for Alzheimer's disease."

Pivotal trials are under way for ganenterumab, a monoclonal antibody for Alzheimer's and one of the most anticipated medications of 2022. The ongoing 4-year Skyline trial will test the new dose in both patients with early Alzheimer's and in those without symptoms but with evidence of plaque accumulation in the brain.[37]

**Neurology medication to watch**

| | |
|---|---|
| **Medication** | Gantenerumab |
| **Company (in Canada)** | Roche |
| **Expected Indication** | Alzheimer's disease or people at risk of the disease |
| **Distinctive feature** | Designed to remove amyloid plaques, one of the characteristic morphological features of Alzheimer's, from the brain.[37] |
| **Newsworthy data** | In an interim analysis of a PET study, gantenerumab (at doses up to 1,200 mg) was associated with robust reductions in amyloid plaque at 2 years.[38] |
| **Other** | Pivotal trial results are expected in the second half of 2022.[37] |

Promising diseases under the neurology umbrella also include Parkinson's and migraine, both common conditions with significant unmet needs. Many people with migraines are unable to use or tolerate currently available treatments, and the disease burden remains high even in those receiving migraine-specific medications.[39] With Parkinson's disease, the greatest unmet need lies in symptomatic motor control.[40]

Here in Canada, innovation in neurology just got a boost with new research collaboration between the Montreal Neurological Institute-Hospital, Takeda Development Center Americas, and Roche. The initiative will harness patient data toward the search for promising targets for neurologic drug development.[41]

> For many years, we couldn't diagnose Alzheimer's until someone died. Being able to identify Alzheimer's in *living people* has opened up *huge treatment doors*.
>
> **Sara Manning Peskin,** Assistant Professor of Clinical Neurology, University of Pennsylvania

**Immunology**

A handful of diseases involving the immune system, such as psoriasis, atopic dermatitis, and asthma, have been driving innovation in immunologic treatments. In the 5-year lead-up to 2026, the market is expected to grow by 39%.[1]

Many of the new treatments for auto-immune disorders are biologics – drugs derived from living material that interrupt faulty signals in the immune pathway. A noteworthy new product in this class, Tezspire, takes aim at the severe form of asthma, which affects 10% of people with the disease.[42]

The strength of the biologic market speaks to the unfortunate prevalence of auto-immune diseases in Canada. Asthma alone impacts more than 3.8 million Canadians, including 850,000 children under age 14, making it the country's third most common chronic disease.[43] Over 300 Canadians receive an asthma diagnosis every day, and asthma attacks kill an estimated 250 Canadians every year.[43]

**Immunology medication to watch**

| | |
|---|---|
| **Medication** | Tezspire (tezepelumab-ekko) |
| **Company (in Canada)** | AstraZeneca |
| **Indication** | Severe asthma (age 12+) |
| **Distinctive feature** | First-in-class biologic for severe asthma that acts at the top of the inflammatory cascade by targeting the epithelial cytokine TSLP[44] |
| **Newsworthy data** | 86% reduction in annual exacerbation rate in patients with nasal polyps and 52% in those without nasal polyps (when added to standard of care, compared to placebo)[45] |
| **Other** | Currently under aligned review by Health Canada and HTA[46] |

The only direction for this market is up, not least because of the steady flow of biosimilars – molecules designed to work like a previously approved biologic – into the mix. Even before an originator biologic loses exclusivity, manufacturers begin working on matching biosimilars, ready to launch them when the patent protection expires. Three of the top-earning biologics in Canada – Remicade, Humira and Lucentis – have already passed through this stage, with Eylea and Stelara next in line.[47] Because biosimilars cost less to develop, they create savings that can be reinvested into innovative new drugs. The next few years should prove very interesting.

## ADAPTING TO THE INFLUX

R&D is just one step in the circle of specialty drug innovation. To complete the circle, stakeholders need to lay down the infrastructure to support new treatments and ensure their sustainability. Suzanne McGurn, CEO of CADTH, welcomes the challenge. "What could be more exciting than figuring out how we distill the promise of these drugs?" she said in an interview with Global Forum, adding that "the HTA process is about providing people with information to decide whether and what to buy, and sometimes it's also about helping them prioritize who should get what and when."[48]

As an example of infrastructure innovation, Canada is stepping up to bring CAR-T therapy to patients in need. Dr. Kevin Hay of the Vancouver Coastal Health Research Institute and his colleagues have received funding to build a Canadian CAR-T manufacturing network and transfer their know-how to other cities.[49] In fact, Toronto and Winnipeg are developing the capacity to manufacture CAR-T cells. The complexities of CAR-T require national collaboration and dedicated centres of excellence, and the collective interest in getting such centres up and running bodes well for the future of CAR-T in Canada.

**Patient support in flux**

In step with such creative collaborations, the patient support program (PSP) ecosystem will need to accommodate the new generation of specialty medicines. PSPs for oncology drugs alone currently hover at 175,[50] and the number of PSPs continues to rise every year. What's more, most existing PSPs support relatively small patient populations – by one industry expert's estimate, no more than 30,000 and often well below that figure.[11] With the market poised to introduce specialty drugs that serve much larger populations, manufacturers and the specialty service providers that support them will need to develop PSPs that can effectively serve these broader and more varied groups.

Technology can help in this regard, enabling the design of more flexible programs that allow patients to customize their PSP journey. For example, today's PSP may have a nurse calling every patient at predetermined intervals. The PSP of tomorrow may enable patients to "choose their own adventure" – their preferred method, frequency, and style of communication.

This flexible, patient-centred philosophy must also extend to caregivers, who play an indispensable role in managing high-need groups such as children, cancer patients, and people with dementia. Indeed, the usual trajectory of dementia has a caregiver stepping up to manage the patient, and as many as 20% of Canadians have experience caring for someone with dementia.[9] Stakeholders increasingly recognize the caregiver journey as integral to the treatment experience, and many PSPs include caregivers in their offerings. Future PSPs have an opportunity to build on this approach to address an expanding array of caregiver needs.

As treatments become increasingly complex, the PSP paradigm may even shift from the individual patient and caregiver to the family unit. For example, a PSP could assist a family that needs to accompany a patient travelling for an infusion, or provide mental health services to help a family cope with the stress of a taxing treatment protocol.

Physicians, for their part, will need to adapt to a prescribing landscape dotted with PSPs. With specialty drugs now entering therapeutic areas historically reserved for general medicine, many specialists who don't currently deal with PSPs will need to learn the ropes. For example, prostate cancer, an area traditionally treated by in-hospital oncologists, is increasingly falling to urologists to manage. With all the specialty migraine drugs entering the market, neurologists may face similar pressures. PSP program administrators will need to educate these clinicians about the nuances of the specialty medicine ecosystem to ensure their patients don't miss vital opportunities for clinical support, prior authorization, or co-pay assistance.

**Brave new data**

Big drugs require big data. Real-world data (RWD) sources such as health-system records and registries can help manufacturers identify the patient population served by a treatment and find out what they're currently missing. In practice, this can pose challenges. For example, current administrative data sets do not code "mild cognitive impairment" as dementia,[11] making it harder to track down candidates for an early-dementia treatment without resorting to labour-intensive chart reviews. Likewise, current data sets do not readily distinguish chronic from episodic migraine patients.[11]

With regulators and payers insisting on increasingly robust evidence to support approval and listing, manufacturers will need to create data plans that span several years – including prior to drug launch. This endeavour may call for creative partnerships, such as the December 2021 agreement between Janssen Research & Development and EVERSANA, a provider of next-gen data services to the life sciences industry. The collaborators will mine data from EVERSANA's chronic disease database, which covers more than 80 million patients, to help enhance clinical trial design and assess how therapies perform in real-world settings.[51]

Data also offers a path toward greater treatment equity. For example, clinical trial data has uncovered the uncomfortable truth that Black people represent only 2% of subjects in Alzheimer's clinical trials, despite developing the disease at higher rates than Caucasians.[52] As the industry zooms in on real-world performance, we have a responsibility to use data to characterize real-world diversity and recruit subjects that reflect it.

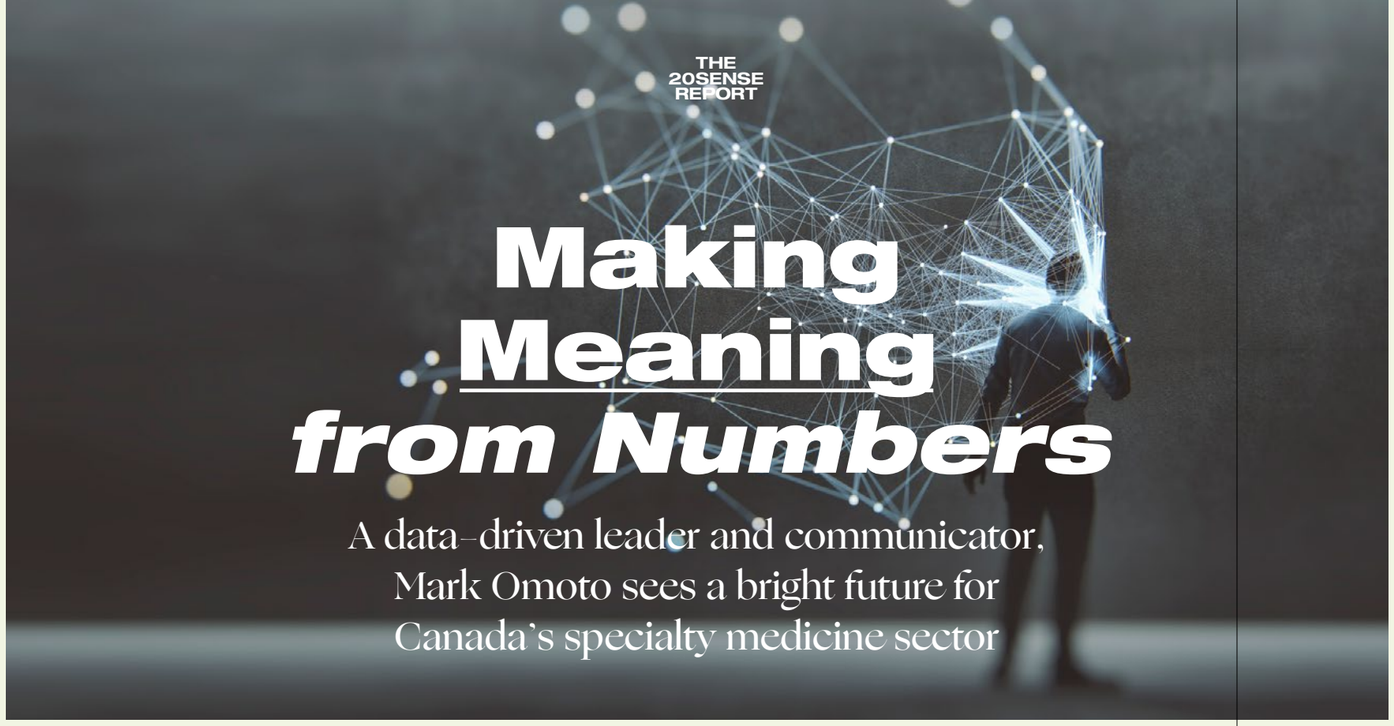
**The way forward**

As the curve of innovation continues to rise, the specialty drug ecosystem will need to evolve apace. Stakeholders throughout the world are setting up new processes to shorten the time to specialty drug access, giving Canada the means to follow suit.

One such initiative, a collaborative global program called Project Orbis, has already made strides toward its goal of accelerating access to innovative cancer treatments. Last year, the program helped fast-track a medication called Tagrisso toward regulatory approval for a new indication – early stage non-small-cell lung cancer – and listing on the same date in the UK.[53] Here in Canada, one of Project Orbis's participant countries, the trek to Tagrisso access hasn't gone quite as quickly: while the new indication received approval in early 2021, the listing application remains in "active negotiation" status at the pCPA as of mid-2022.[54]

As the above case exemplifies, access will likely pose a challenge for some time to come, especially for therapies that show promise but have limited evidence and a high degree of budget uncertainty. The pipeline abounds with such medications, offering new hope to patients – but only if they can access the drugs. As the innovative specialty medicine pipeline evolves, stakeholders will need to explore innovative approaches that harness real-world evidence to mitigate risk. Specific strategies and tools may change as the pipeline diversifies, but the goal remains the same: the right medicine to the right patient at the right time.

> What could be more exciting than figuring out how we distill the promise of these new treatments?
>
> **Suzanne McGurn, CEO, CADTH**



THE 20SENSE REPORT

# Making Meaning from Numbers

## A data-driven leader and communicator, Mark Omoto sees a bright future for Canada's specialty medicine sector

**As General Manager at IQVIA Canada, the country's leading provider of healthcare data analytics, Mark Omoto oversees IQVIA's thought leadership, marketing and communications strategy and provides consulting expertise to companies in the healthcare and other sectors.** Mark's deep experience in the pharmaceutical industry has seen him involved in product launches, market access, and global marketing. A popular keynote presenter at industry conferences, Mark pressed pause on his busy schedule to share his insights about the specialty medicine pipeline.

**In a nutshell, what does IQVIA do?**

We're the largest global provider of advanced analytics, technology solutions, and clinical research services to the life sciences industry. You can think of us as a mirror to the pharmaceutical industry: we provide products and services throughout the drug life cycle, with a focus on advanced data and analytics.

**How do you define a specialty pharmaceutical product?**

The term doesn't have an official definition, though a lot has to do with complexity and cost. Generally speaking, specialty pharmaceuticals treat chronic, complex, or rare conditions. They may require special handling in the supply chain, have highly specific distribution pathways, or require extensive monitoring. We sometimes use $7,500 per year as a loose lower cost threshold. Within specialty, we often divide medications into biologics and non-biologics.

**How would you describe the specialty innovation we've seen in Canada over the past decade?**

We're in the midst of a shift from infused and injected products toward oral medications, notably in oncology and auto-immune diseases. We're also seeing an expansion in the therapeutic areas covered by specialty medications, including the addition of new indications for established products. For example, Dupixent, initially indicated for atopic dermatitis, now has an indication for asthma. Diabetes medications are now receiving indications for obesity. In essence, specialty drugs are moving into the primary-care sphere. They're also being initiated earlier in treatment algorithms.

**How is Canada doing in terms of specialty medication innovation and adoption?**

I think we're in a golden age. The medications available today give Canadians more options than ever for managing both chronic and acute conditions. In both 2020 and 2021, specialty pharmaceuticals accounted for over 33% of all new active substances commercialized in Canada. And thanks to our public and private coverage ecosystem, we're seeing very robust uptake of biologics across many therapeutic areas.

**How has the COVID-19 pandemic impacted the evolution of specialty medicine in Canada over the past couple of years?**

Average sales performance for new active substance product launches across the globe during the pandemic are down by 19% from the pre-COVID benchmarks. This is to be expected, as the pandemic had affected every corner of medicine. At the same time, our recent experience with the COVID-19 vaccines speaks to how well and how fast the pharmaceutical industry can innovate when all stakeholders are aligned.

The pandemic has also raised our collective awareness of mental health and the challenges facing long-term care homes. So we're now seeing a renewed interest in treatments for neurologic conditions, especially Alzheimer's disease. During the course of the pandemic, the neurology market has doubled its rate of growth, which means more patients than ever will have access to effective mental health treatments – one of the few positive things to come out of the pandemic.



> Innovation is going to come *faster and faster*, and new products will keep getting more complex.

**Where do you see the specialty pipeline going?**

What we've seen so far is just the beginning. Innovation is going to come faster and faster, and new products will keep getting more complex. The big question going forward will be: how much improvement does the innovation provide, and will it be worth it? Like everything in life, specialty drug development requires us to consider tradeoffs.

**What are the key specialty areas to watch for?**

Given the phenomenal rate of innovation in oncology, diabetes and immunology, we're keeping a particularly close eye on those areas. The oncology pipeline alone is full of new medications for increasingly specific indications. It's telling that rare cancers account for 40% of the oncology pipeline. We're seeing a lot of immune checkpoint inhibitors and antibody drug conjugates coming through, and the success of biotherapies such as CAR-T bodes well for this category. The increasingly personalized approach to cancer treatment has also fuelled an interest in combining medications to optimize outcomes in different subgroups.

Neurology represents another area of high need. Right now, 154 molecules are being investigated for Alzheimer's – some biologics, some cell therapies, some small molecules. It's a complex area of investigation, with a high probability of failure, so the high research interest speaks to the urgency of the public need. R&D for Parkinson's disease, depression, multiple sclerosis, migraine, and epilepsy is also thriving.

**How will the Canadian specialty infrastructure need to adapt to support all the innovation?**

By and large, our specialty infrastructure has been able to support the needs of patients on injected and infused drugs. We now have to integrate biotherapeutics, like CAR-T therapies, and other next-generation treatments into the system. Many of these therapeutics don't fit into the traditional clinical and pharmacy distribution pathways. To build the required infrastructure, the private market will need to form partnerships with the public sector.

> To build the infrastructure to support biotherapeutics and other next-generation treatments, the private market will need to form partnerships with the public sector.

**Has IQVIA had to evolve to meet the new needs associated with these therapies?**

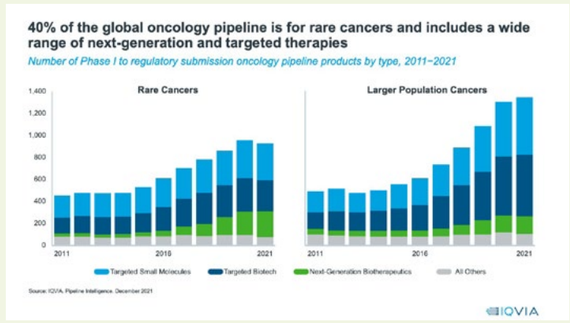
Yes. In addition to tracking market segments for brands, generics, and biologics, we have added a speciality classification to our insights. We also track distribution channels beyond hospitals and pharmacy to reflect the increase in non-traditional distribution channels. For example, mail order pharmacies offer home delivery to patients, so we monitor this channel.

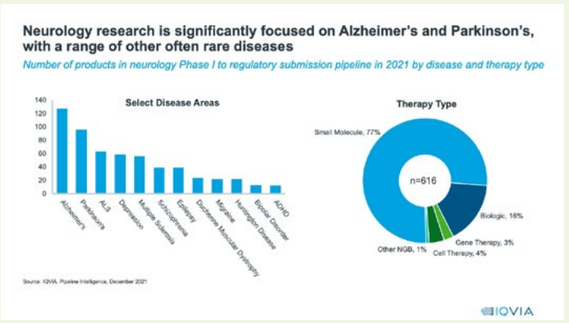
**Do you expect some of the pipeline drugs to present challenges from a data perspective? How do you see the role of real-world evidence (RWE) in filling data gaps?**

RWE has evolved by leaps and bounds, to the point that regulatory bodies are routinely incorporating it into their decisions. There's still room for improvement in capturing data across multiple therapeutic areas and jurisdictions. Perhaps the biggest challenge lies in achieving consensus on which data matters most, especially for a pipeline drug that doesn't have a precedent. I'm confident we can get there.

**What are you most excited about in today's specialty drug market?**

Whether you're a patient, healthcare professional or industry stakeholder, there's a lot to be excited about. Innovations are reaching the marketplace at an incredible pace. It's especially exciting to see specialty medicine confront the many chronic diseases that impact our aging Canadian population. Of course, benefits don't come without costs. It's all about striking the right balance. I hold onto the thought that innovation will continue to improve Canadian lives.



# On the reading *list*

Breast cancer drug trial results in 'unheard-of' survival

The Montreal Neurological Institute-Hospital Genomics Partnership aims to drive neurological disease research

Alzheimer's trials exclude black patients at 'astonishing' rate

'Of Medicine and Miracles' review: Immensely moving documentary finds hope in the fight against cancer

What if Alzheimer's is an autoimmune disease? Toronto neurologist awarded for work on unconventional hypothesis

Exploring diabetes and stem cells: How close are we to a cure?

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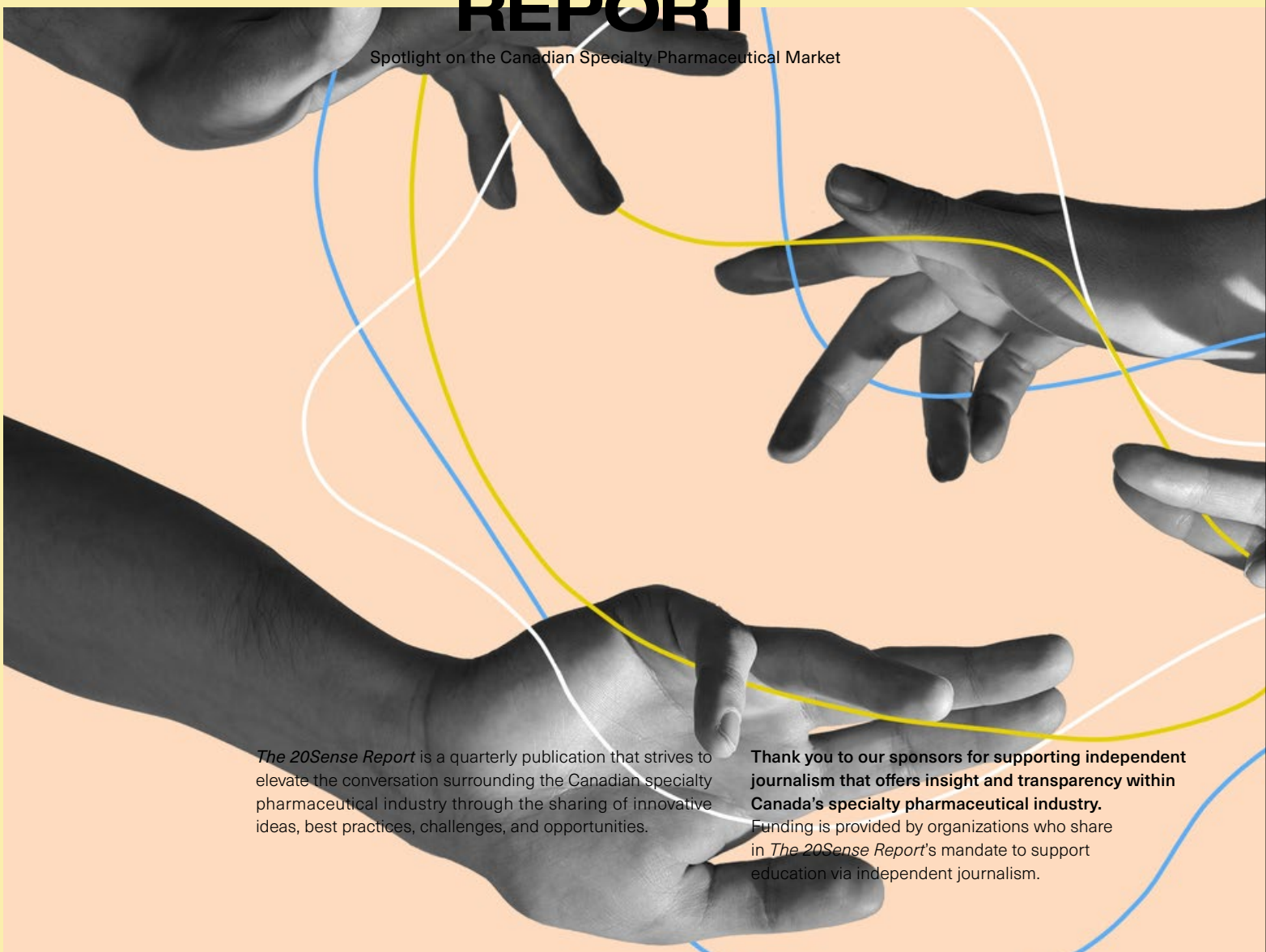
# THE 20SENSE REPORT

Spotlight on the Canadian Specialty Pharmaceutical Market



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Inquiries may be directed to
**info@20Sense.ca**
**www.20Sense.ca**
LinkedIn