

THE 20SENSE REPORT

SPOTLIGHT ON THE CANADIAN
SPECIALTY PHARMACEUTICAL MARKET

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Making sense of Canada's
specialty pharmaceutical market



From Patients, for Patients



Patient-centric data can help
us use specialty medications
more effectively

Using Patient Data to Improve Patient Care: Are We There Yet?

Specialty medications continue to conquer new ground, becoming more sophisticated and widespread with each passing year. Between 2018 and 2019, use of these medications among Canadians grew by 7.8%,¹ and growth continues apace. These drugs take a lot of effort to develop, but they save and transform lives.

And yet.

These “modern miracles” aren’t consistently reaching the Canadians who need them. In a stakeholder survey conducted in 2018-19, both Canadian cancer patients and their physicians identified “long waits and inaccuracies in diagnoses” as top issues in oncology treatment.² The next hurdle is access. One in five Canadians struggles to pay for prescription medications,³ and the path to reimbursement for specialty drugs has become so tangled that few patients can travel it on their own.

On the plus side, stakeholders are getting serious about using patient data to tackle these problems. A white paper called *Mission: Patient Centricity* explains how outcomes data can help demonstrate treatment efficacy, support negotiations with payers, and keep patients with rare diseases motivated.⁴ Patients themselves have joined forces to identify and communicate the health outcomes that matter to them. The initiatives detailed below suggest we’re on the right track; we just need to connect the guideposts.

Patients seeking value

No longer content to sit on the sidelines, patient organizations are setting the agenda for the selection and use of real-world data. After the concept of value-based healthcare (VBHC) gained traction in a 2018 summit, the patient group Coalition Priorité Cancer au Québec saw an opportunity to mobilize VBHC in the province. Partnering with VBHC Canada, the Coalition launched a patient-led demonstration project to review

patient-reported outcome measures (PROMs) for breast, lung and colorectal cancer.⁵ The initiative entrusted patients in four cancer care centres to validate the outcomes and identify obstacles to achieving them, while keeping all levels of government abreast of their progress.⁵ If all goes according to plan, the learning will give rise to policies and procedures that better support the PROMs.

Along similar lines, Colorectal Cancer Canada (CCC) has launched the Patient Values Project to assign weights to various patient priorities in cancer drug treatment.⁶ These weights will give other patient groups a new vocabulary for liaising with governments and increasing timely access to the cancer drugs that best meet their needs.⁶ At the same time, CCC is pushing for the collection of patient-centric data in clinical trials.⁶

As a shining example of the narrowing gap between ivory tower and ground-level reality, the Canadian Myeloma Research



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Network has set up a database of real-world outcomes that researchers and governments can use to explore therapeutic gaps and solutions.⁷ The network's goals also include dialing up the patient voice in clinical trial steering committees and facilitating access to new treatments that are not yet reimbursed.

First-hand experience

In tandem with these developments, patient experience is steadily gaining ground as a metric to consider in drug development. To this end, various patient groups are polling their members to find out exactly what they like and dislike about their condition and its treatment.

In a recent virtual symposium, the Canadian Skin Patient Alliance presented results of its survey of patients with hidradenitis suppurativa, a painful skin condition affecting up to 4% of Canadians.⁸ Attesting to the enormous challenge posed by this disease, survey respondents had tried an average of 15 treatments, surgeries and lifestyle modifications.⁹ And while 36% used biologics to manage the condition, only 20% reported satisfaction with currently available treatments, reflecting a considerable unmet need.⁸ Similarly, Migraine Canada is developing surveys to capture the experience of living with migraines and gathering testimonials from people who have tried different treatments for the disorder.¹⁰

The Canadian Agency for Drugs and Technologies in Health (CADTH) takes such data points seriously. At the outset of a project, CADTH solicits feedback from patients – typically centred on disease impact, treatments, and presentation of information – and folds the input into its drug reviews.¹¹ In an analysis of this process, CADTH concluded that “patients offer unique insight into what it is like to live with a specific disease or medical condition; they also help by sharing their hopes for new treatments.”¹¹

Distinct and bold

Sharing data with regulators is one thing, but what about industry? The idea made sense to Pierre Fitzgibbon, Quebec's Minister of Economy, who recently suggested giving the pharma industry access – at a cost – to patient data held by the Régie de

Patients get a hearing: the case of Aimivog

In July of this year, CADTH's Canadian Drug Expert Committee (CDEC) recommended that the specialty migraine drug Aimivog (erenumab) be reimbursed for patients meeting specific criteria.¹² The recommendation represented the culmination of an arduous review process that included two reconsideration meetings.¹² Patient groups can take at least part of the credit for this outcome, having identified an unmet need for safe and effective medications to prevent chronic migraines. When making its final recommendation, CDEC considered input from Migraine Canada and Migraine Quebec, including a national patient survey affirming that migraine impacted intimate and family relationships for 97% of respondents.^{12,13}

l'assurance maladie du Québec (RAMQ).¹⁴ Stating that the future of medicine lies in using data to customize treatment, he maintained it was possible to share the data with industry without breaching patient confidentiality.¹⁴

As expected, not everyone cheered. Solidarity deputy Vincent Marissal, for one, likened the move to giving “the keys to the bloodbank to the vampires.”¹⁴ The Alliance des patients pour la santé took a more nuanced position. In a social media post, the group affirmed that “the road turns” and “there is no point in sticking your head in the sand.”¹⁵ Noting the theoretical benefits of the idea to research and patient care, they deemed it worthwhile to explore the feasibility of the proposal.¹⁵

Measures that matter

Gone are the days when a drug's efficacy in clinical trials trumped all other considerations. The success of today's specialty medications depends increasingly on data from the real world. No longer an afterthought, such real-world data is helping to shape everything from drug development and evaluation to PSP design and agreements with payers.

As a growing number of studies have shown, clinical trial data does not reliably translate to broader populations. A case in point: an analysis of 15 studies published this year, researchers compared the effect of riluzole, a drug for amyotrophic lateral sclerosis (ALS), in population studies versus clinical trials.¹⁶ As it turned out, the drug extended lives by 6 to 19 months longer in real-world

situations than in clinical trials, giving hope to ALS patients and strengthening the rationale for the medication.¹⁶

Real-world data can inform not just treatment, but diagnosis. In one instance, Quebec and Ontario researchers analyzed tissue samples from 483 prostate tumors, identified the “molecular signature” of each sample, and used the information to develop algorithms for recognizing different forms of prostate cancer.¹⁸

Industry, for its part, has been harnessing the PSP infrastructure to collect real-world evidence (RWE) to support research and improve services in these programs. To get a pulse on this trend, the RWE & OBA Working Group surveyed Canadian pharmaceutical companies with PSPs and PSP vendors in July 2020.¹⁷ Questions focused on the current status of PSP data collection and the potential for RWE to support outcomes-based agreements (OBAs) with payers. Results will be published late this year.

We're getting close

The pieces are all in place. Canada has shown itself capable of using data as a diagnostic and treatment tool, to identify outcomes that mean the most to patients, and to amalgamate the patient perspective into decisions about specialty drugs. Just a few more bold moves will take us to the finish line: using patient data to assess specialty drug performance and get the right drugs to the patients who need them. **If we continue to put patients first, we'll get there.**



Patients, Projects, and Data Points: Eva Villalba Connects the Dots

It's not for nothing that Eva's colleagues call her a "dot connector." As Executive Director at The Quebec Cancer Coalition, an umbrella group of more than 60 non-profit cancer organizations in Quebec, Eva constantly looks for opportunities to catalyze partnerships and joint projects. On the volunteering circuit since age 12, Eva spent more than a decade working in palliative care, where she cut her teeth on policy work. Along the way, she obtained an MBA and helped to shape public policy in oncology through her involvement with patient advocacy groups. Here, Eva applies her sharp mind and big heart to the challenge of collecting and using patient data in Canada.

Q: How is the Coalition helping to catalyze the use of patient data?

We see our role as raising awareness of the need for patient data. We have been pushing the government to update our current Quebec Cancer Registry, which is 8 years behind in its data. We would like the registry to be as up-to date as those in Ontario or Alberta, to include predictive data, and to be accessible to researchers and decision makers. We are also big fans of value-based healthcare (VBHC) as a way to modernize cancer care in Quebec and make decisions based on what's best for the patient, not only the system. Patients don't want more healthcare, they want more health.

Q: How do patients feel about sharing their data?

It all depends on how you broach the question. If you ask them to share their data so it can be sold to industry, they will say no. But if you frame the request as an opportunity to help patients and society,

99 percent will gladly agree. Of course, you have to assure them of complete confidentiality.

Q: How does VBHC intersect with cancer treatment?

Meaningful treatment outcomes go far beyond progression-free survival: for example, one patient may be concerned about preserving fertility, another about preventing incontinence. A VBHC approach ensures these personal priorities are included in the treatment pathway. This year, in collaboration with The Conference Board of Canada, we will be looking at how VBHC can improve outcomes in colorectal, lung, and breast cancer.

Q: How could VBHC benefit treatment at a systems level?

Some of the most effective medications on the market are currently offered third-line, after a patient has "failed" standard treatments. RWE would help quantify potential problems with this stepwise paradigm,

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which can cause a drain on resources, lost productivity from extra hospitalization, and increased individual suffering. In some cases it may be more cost-effective to use the best medication first, even if it costs a little more. This is something that VBHC can demonstrate.

Q: Tells us about your research study on PROMs.

We will be conducting patient focus groups using a method that encourages free expression. We plan to divide patients into segments, which is very important because even among the same cancer type, different populations (for example: different age groups, stages of cancer, rural/urban dwellers) have different needs. We will communicate the results to patient associations and regulatory bodies so they know which outcomes are most important to patients. For example, fatigue tends to be very debilitating in ovarian cancer and should be included as an outcome in ovarian cancer trials.

Q: Should patient support programs (PSPs) be collecting patient data?

PSPs are ideally positioned to collect data as they have regular contact with patients. I support a model in which such data should be shared with public authorities so it benefits all patients. Some PSPs have begun collecting quality-of-life data, but the practice is still in its infancy. I think manufacturers will be willing to invest more in this area when they have clarity that regulators will consider patient data in their drug assessments.

Q: What roadblocks, if any, are preventing patient data from being used to its full capacity?

Quebec has a highly entrepreneurial private sector, but the public sector has been slower to embrace change. The COVID-19 crisis has proven that we can act fast when we need to, such as setting up telehealth in a hurry. If we can do it for COVID, we can do it for cancer.

Q: The Quebec Minister of the Economy recently announced the possibility of giving industry access to RAMQ data. Is this a good idea?

The confidentiality issue wasn't properly addressed in the announcement, so many people reacted negatively. That was unfortunate, because access to RAMQ data could lead to better clinical trials and treatment decisions. For example, we may learn that patients on a certain immunotherapy survive X months or years longer than on standard treatment. Hopefully the government will communicate more clearly about the proposal going forward.

Q: Does it make sense to evaluate specialty medicines based on real-world outcomes?

Yes. The precision treatments being developed today often target very small populations, making it difficult to conduct phase 3 clinical trials. It makes more sense to allow conditional drug approvals based on RWE from patients with special access. We are encouraging the government to look at this paradigm more seriously.

Q: How do you see health outcomes data evolving over the next five years?

We would like to see RWE tied to reimbursement as we believe such risk sharing helps ensure the fairness and sustainability of the health system. Other parts of the world, such as Australia and Europe, are further ahead in this regard. We don't lack the expertise here in Canada – we just need the political will to make it happen.

“It may be more cost-effective to use the best medication first, even if it costs a little more. This is something that RWE and value-based healthcare can help demonstrate.”



Handle With Care: Lisa Machado on the Wise Use of Patient Data

Twelve years ago, after a life-changing diagnosis of chronic myeloid leukemia (CML), Lisa shifted her career focus from financial journalism to healthcare advocacy and education. In 2014, she founded the Canadian CML Network, a non-profit organization working with CML patients and their healthcare providers throughout Canada. Early this year, Lisa became the Executive Producer at healthing.ca, a website that helps patients optimize their health in the midst of chronic disease. In this candid interview, Lisa shares her nuanced views about patient data.

Q: What led you to become a patient advocate?

Before my diagnosis of CML, I had a black-and-white view of cancer: either you die of it or you get cured. But I learned that cancer means uncertainty – advocacy was my way of taking back some control in my life. As I reached out to more people, I realized I wasn't the only one struggling. One thing led to another, and I began participating in policy meetings to explore how people live with rare diseases. This work has helped me move forward.

Q: Everyone is talking about patient data these days. Do you think it is being used correctly?

We still have a way to go. For example, patients aren't always kept abreast of how their data is being used, which is a missed opportunity to build trust. Real-world data also needs to reflect the socioeconomic and ethnic diversity of the patient population being served, and this isn't always happening.

Q: How do you feel about sharing your own data with healthcare stakeholders?

On the one hand, I think it's great that my experiences are being used to improve the care of others. But I also worry that the information will be shared in a way that isn't helpful. And privacy is always a concern, of course.

Q: Is privacy an issue for most patients?

Yes. Many people worry they won't get hired or will be laid off, for example, if word gets out that they have an "expensive" disease. Others hesitate to share information with PSPs, such as side effects, because they fear support of their treatment may be stopped if they complain.

Q: How might the industry address such fears?

Transparency is key. It should be made clear that any personal data will be de-identified and never be used against them. That's not to say that everyone will be convinced, but

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there is definitely room for the industry to have more open conversations to gain trust and foster confidence.

Q: What do you think about measuring a drug's performance based on patient health outcomes?

Once again I'm of two minds. Data from EMR records can help us decide where to invest our healthcare dollars, and real-word data can give a boost to high-performing medications. I fully support these ideas. At the same time, I worry that data could be used to limit access to drugs that benefit only a small pool of people. If the drug works, we should be funding it.

Q: How would you hope your data would be used?

Ideally, my data would be used to show that taking a chance on an expensive drug can not only make lives better, but also make important differences elsewhere. For example, some drugs for CML are now being used for other diseases.

Q: What is the value of PROMs?

PROMs give us valuable insights into what it means to live with a disease. You might learn that many patients on a certain drug get up every night or have panic attacks, which you wouldn't necessarily discover from clinical data. Used intelligently, PROMs can help us figure out how to improve patients' quality of life.

“We have to ensure that the health outcomes data we collect is being used to save lives. That's the goal.”

Q: What role do PSPs have in collecting data?

PSPs are a natural vehicle for data collection because they have a built-in patient pool. That said, I would like to see PSPs do more than passively collect data from patient surveys. In my experience, exploratory one-on-one or group conversations yield higher quality feedback than surveys. Industry and their PSP provider partners have an opportunity to engage patients as active partners whose feedback helps improve the treatment experience.

Q: What is preventing patient data from being used more extensively and holistically within the specialty medicine ecosystem?

I come back to transparency. A while back, the Canadian Medical Association surveyed patients on their attitudes about data. The patients did not object to sharing data on principle – they just wanted to know how it would be used and by whom. Patients want to know what happens after they agree to “donate” their data. That's the missing piece.

Q: What do you see as the future for health outcomes data?

I hope to see this area grow. Not a week goes by when we don't hear about a rare disease that needs drug funding. As we move forward, we have to ensure the data we collect is being used to save lives. That's the goal.

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Patient-Centred Initiatives You Need To Know About

Patients are speaking and the healthcare system is listening. This sampling of initiatives, either led by or focused on patients, highlights the rising chorus of Canadian patient voices and the increasing prominence of patient data within the specialty medicine ecosystem.

One-stop shop: Drug Access Canada is born¹⁹

As befits its name, Drug Access Canada (DAC) is all about patient access to specialty treatment, focusing on Drug Access Navigators to get the job done. Having watched the profession come into its own over the past decade, DAC determined that navigators increase patient satisfaction and take the administrative burden off health providers so they have more time to care for patients. The group's database keeps health professionals abreast of currently available specialty medications and corresponding patient support programs (PSPs), at no cost. A task force to improve and streamline the Canadian PSP ecosystem is in the works.

Uncommon leadership: CORD steps up training for patients with rare diseases^{20,21}

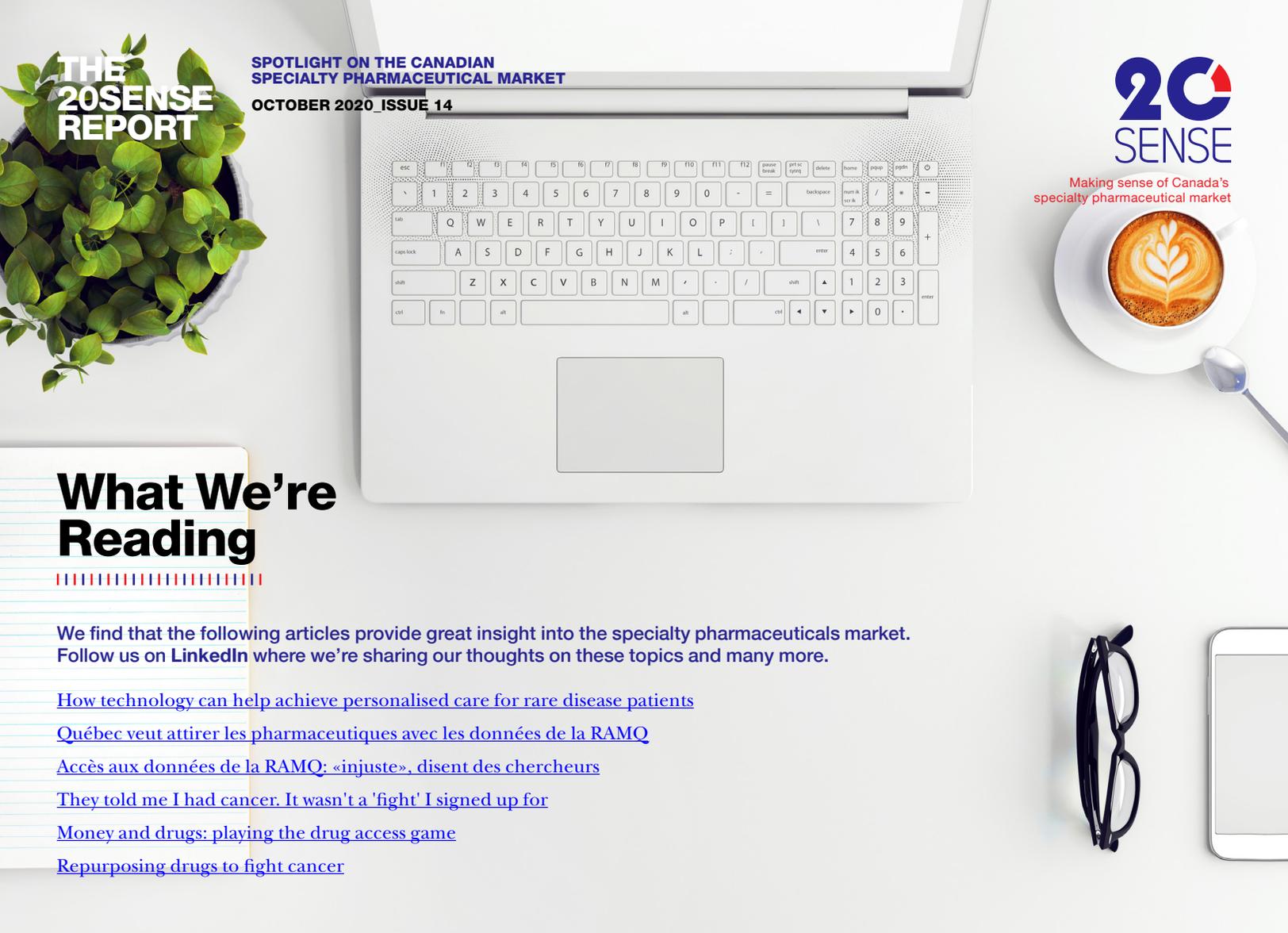
Many people become advocates in the trenches, often as a result of a major life challenge – but advocacy skills can also sharpen through instruction. To this end, the Canadian Organization for Rare Disorders (CORD) launched Co-Creating Highly Effective Patient Partnerships, a leadership training program geared to patient advocates, in the fall of 2020. Featuring international speakers and interactive sessions, the 10-week course covered the contribution of patients to rare drug discovery, the role of the patient at each phase of the drug lifecycle, the importance of patient-relevant outcome measures, and patient engagement in the regulatory process.

Calling the shots: Patients set the agenda in the Save Your Skin summit⁵

In late 2019, for the fourth consecutive year, the Save Your Skin Foundation and other patient groups hosted an annual event called Patients Redefining Healthcare Summit (formerly Drug Pricing Policy Summit). Over the years, this meeting of minds has spawned several working groups and projects focused on PROMs and patient data. The 2019 meeting took a deep dive into value-based healthcare (VBHC) and patient-reported outcome measures (PROMs), with indigenous health and social determinants of health receiving special attention. In a breakout session, participants explored strengths and gaps in population- and disease-specific PROMs.

A large pond to swim in: CHORD pools Canadian cancer data²²

The Cancer Health Outcomes Research Database (CHORD) consortium exists for one reason: to collect and share cancer data for research. This national initiative, which brings together cancer researchers across the country and includes data provided by PSP providers, is proving especially helpful in research on rare and complex tumors, for which single institutions may lack adequate sample sizes. The effort has borne fruit in several research initiatives, including a 2018 study to help predict survival in hepatocellular carcinoma (HCC) patients treated with sorafenib. The database enabled the researchers to estimate the prognosis of HCC patients using five different staging models.



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.

[How technology can help achieve personalised care for rare disease patients](#)

[Québec veut attirer les pharmaceutiques avec les données de la RAMQ](#)

[Accès aux données de la RAMQ: «injuste», disent des chercheurs](#)

[They told me I had cancer. It wasn't a 'fight' I signed up for](#)

[Money and drugs: playing the drug access game](#)

[Repurposing drugs to fight cancer](#)

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 treatments
- Oncology therapies in the pipeline

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.

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