Advancing Cell & Gene Therapy

What We Heard

On September 30, 2024, Advancing Cell & Gene Therapy held a virtual round table exclusively focused on patient groups in Quebec. Committed to a common objective, patient organizations shared their main expectations in terms of information, training and support. They outlined the very real consequences that could result from delayed access to treatment, revealed systemic challenges – such as the system's capacity shortcomings and interprovincial inequalities – and advanced tangible proposals for improving the management of GCT.

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This virtual round table provided a key opportunity to integrate the patient's voice into strategic thinking about the deployment of GCT in Quebec. The objectives were to:

1. **Identify the priority needs** in terms of information, training and support for patients.
2. **Hear direct testimonies** about the tangible impact of delayed access to GCT.
3. **Highlight structural issues** in Quebec's health care system, namely its limited capacity and interprovincial disparities.
4. **Propose activities** adapted to patients' realities, ranging from reforming assessment processes to creating satellite centres to improve geographic accessibility.

Organizations that attended the virtual roundtable:

- Best Myeloma Canada
- Association de la neurofibromatose du Québec (ANFQ)
- Association de l'anémie falciforme du Québec
- Sickle Cell Disease Association of Canada
- Canadian Organization for Rare Disorders (CORD)
- Canadian Hemophilia Society (SCHQ)
- Regroupement québécois des maladies orphelines (RQMO)
- MS Canada
- Lupus Canada (CIAN)
- Parkinson Québec

Hope for the future

- Cell and gene therapies, in particular for blood cancers and certain rare disorders, provide cause for optimism within the medical community and among patients thanks to proven clinical results. These medical advances not only provide a paradigm shift in treatment but also hope for healing or major quality of life improvements, fueling a strong commitment by patient groups to promoting their deployment.

- Patient organizations emphasize the need for sustainable investments in infrastructures and research to build on these early successes. They highlight the fact that tangible collaborations among clinicians, researchers, industry and governments have already borne fruit, and must now be strengthened and solidified.

- A recurring observation is the gap between the speed with which science is evolving and the lumbering regulatory assessment (such as the CDA and INESSS), negotiation and reimbursement processes. These delays risk relegating Canada to the “bottom of the list” of territories where new therapies are being introduced, which is of great concern to patients and clinicians.

- Certain treatments, such as CAR-T therapies, which are already available and routine in the United States and Europe, have yet to become available because of interrupted negotiations or administrative roadblocks. These obstacles are seen as denying access to vital care for patients who, sometimes, have no other therapeutic options remaining.

- Because of specific expertise, current assessment processes do not always appear to be adapted to the innovation those therapies represent. Recommendations stemming from the first assessments may seem inconsistent in light of international data, thereby shaking patients’ confidence in the system.

- Hope remains strong, but is seen as fragile. Stakeholders acknowledge that it depends on the system’s ability to adapt more quickly, to receive adequate financial support and to increase the number of human resources involved in GCT.

Education is essential

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- Patient associations play a central role as intermediaries between complex medical information and patients', families' and communities' understanding. They translate protocols, benefits and risks into clear language so people can make informed decisions about potentially life-changing therapies.

- This effort to simplify the information is complicated by institutions' lack of transparency. When decisions and justifications are not communicated, it becomes difficult for associations to explain to patients why a given treatment is accessible in one country or province, but not in theirs, leading to uncertainty and anxiety.

- Education must also target health professionals, namely those who are not specialists, who may be called to prescribe or refer. Training about health corridors, diagnostics and therapeutic options is deemed crucial because any gap in medical knowledge in these areas directly impacts access.

- The role of patient partners is seen as positive, but is unequally valued. While they may be fully integrated into certain exercises, in others they are simply relegated to "ticking off a box" without being given any real influence over decisions, thereby limiting the educational and collaborative scope of their contribution.

- The limited resources of associations representing patients constitute a major barrier to continuing education programs. Many of them can do nothing more than organize the occasional event due to lack of funding, and are left waiting for the government to invest more in educational initiatives directly related to the adoption of new therapies.

- Integrating education into clinical trials – for both medical teams and patients – is seen as a good way to till the field in preparation for these new therapies. It is an opportunity to identify operational challenges upstream and to strengthen stakeholders' trust.

Access is an issue

- Canada's regulatory process, which involves submitting applications to Health Canada for provincial registration, is deemed excessively long and complex. Although safety is the justification for such thoroughness, it delays the availability of treatments already considered standard elsewhere, and puts at risk the health of patients in need of urgent care.

- The high costs of cell and gene therapies, as well as restrictive reimbursement criteria constitute major barriers to their use. The unwillingness to explore alternative reimbursement models, such as payment by instalments or risk sharing based on clinical trial results, hinders access.

- Geographic disparities exacerbate the obstacles. Patients living in more remote areas often have to travel to urban centres to receive treatment, which means extra transportation and accommodation expenses and lost income. For some people, these obstacles put access to treatment beyond their reach.

- Unexpected hurdles emerge in hospitals when choosing a treatment or therapy. Without access to actual costs, facilities' internal budgets (e.g. pharmacy department) can constrain the use of certain therapies even though these can be negotiated downwards with the government.

- In the absence of dialog during negotiations (with the APP), patients are left out of crucial discussions that impact access. Associations call for more transparency in processes so they can inform their members about the latest breakthroughs, avoid rumours and better guide the choice of treatments.

A need to increase the system's capacity

- Because of the limited capacity of the system and establishments (too few beds, trained professionals and equipment) certain patients are prioritized over others, giving rise to ethical dilemmas. Clinicians do not want to be the only ones to answer the following question: who will receive the treatment when everyone has a vital need?

- The system's capacity to absorb the volume or complexity of treatments could be why therapies are passed over and certain negotiations are unsuccessful. Without more human and material resources, access to these innovations may remain limited.

- To avoid having choices left to the judgment of only clinicians, stakeholders suggest that patients, doctors and decision-makers work together on developing transparent criteria for prioritizing access to treatments when the system does not have enough capacity.

- It is argued that practical training of staff through multicentric clinical trials is essential. Testing procedures in a controlled setting enable teams to acquire expertise and identify the necessary adjustments well before large-scale deployment of therapies.

- An integrated approach to the health corridor, from early detection to post-treatment rehabilitation, must replace current silos. This includes virtual monitoring, devices that can be used in the home, or stronger coordination between urban centres and rural areas.

- The participants insisted on the need to start planning now for the massive influx of new therapies using a funding and adaptive logistics model. It is imperative that factors limiting access and exacerbating existing inequalities be reduced.

Worth Noting

This report, which echoes the voices of patient groups, confirms previous observations and highlights the fact that interprovincial and intraprovincial equity in health care remains a key issue. The priorities can be summarized as three axes: guaranteeing uniform access to innovative therapies across Quebec, reducing assessment and implementation timelines, and strengthening the system's capacity to integrate new therapies. It argues for rapid action to ensure that equity and access become a given for everyone.

About Advancing Cell & Gene Therapy

Established in 2022, Advancing Cell & Gene Therapy is a group of leading Canadian innovative pharmaceutical companies invested in the future of innovation in health care. We are committed to moving forward the discussion on equitable access to cell and gene therapies that could change, even save, the lives of Canadian patients. The members of the group who support this work are: