Disease Modifying Therapies for Type 1 Diabetes

Preparing EU health systems for innovative treatments

European Diabetes Forum

Member Organisations:

EASD

EFSD

FEND
Foundation of European Nurses in Diabetes

Breakthrough T1D™
Formerly JDRF

International Diabetes Federation
Europe

ISPAD
International Society for Pediatric and Adolescent Diabetes

PCDE
primary care diabetes europe

Société Francophone du diabète

Disease-modifying therapies for type 1 diabetes: A call for action

DISEASE-MODIFYING THERAPIES FOR TYPE 1 DIABETES: A CALL FOR ACTION

Type 1 diabetes (T1D) is a lifelong autoimmune condition that can occur at any age and requires daily insulin therapy for survival. However, it is time to shift how we view insulin. While it remains a critical and life-saving treatment, it is not a cure, nor a long-term solution. Insulin therapy enables survival but does not address the underlying disease process. As Starr et al. (2024)[1] argue, the field must move beyond viewing insulin as a sufficient solution and recognize the urgent need for breakthrough therapies that target the root causes of T1D and fundamentally alter its course.

The emergence of disease-modifying therapies (DMTs) marks a pivotal step forward. These therapies aim to prevent, delay, or slow progression by modulating the immune response and bring us closer to a future where T1D is not only treatable, but potentially preventable.

WHY EUROPE MUST ACT NOW

With DMTs already available in some global markets, the European Union (EU), its Member States, healthcare stakeholders, and the broader public must act urgently to prepare for their integration into healthcare systems. Without proactive policy measures, Europe risks falling behind in access, innovation, and public health outcomes.

Timely policy action will reinforce Europe's leadership in biomedical innovation, aligning with the EU's strategic priorities outlined in the Draghi report and strengthening its global competitiveness.

1. Starr L, Dutta S, Danne T, Karpen SR, Hutton C, Kowalski A. The Urgent Need for Breakthrough Therapies and a World Without Type 1 Diabetes. Diabetes Ther. 2025 Jun;16(6):1063-1076. doi: 10.1007/s13300-025-01735-6. Epub 2025 Apr 11. PMID: 40214899; PMCID: PMC12085400.

ABOUT THIS PAPER

This policy paper from the European Diabetes Forum (EUDF) outlines key steps to ensure that EU healthcare systems are prepared to safely and equitably deliver DMTs for T1D. It is intended to support:

- ☑ **Policymakers,** by offering clear and actionable strategies to improve future access to care;
- ☑ **Healthcare professionals (HCPs)** and people living with T1D, by identifying critical adaptations in service delivery;
- ☑ **Stakeholders** across the health ecosystem, by highlighting the regulatory, research, and workforce investments needed to make DMTs a reality.

Key Recommendations

1. **Prepare** healthcare systems and professionals to deliver DMTs for T1D by implementing workforce training and updating care pathways.
2. **Introduce** early detection of T1D through islet cell antibody screening as a standard of care.
3. **Strengthen** research and ensure efficient regulatory pathways to support the development, evaluation, and approval of DMTs for T1D.

Type 1 diabetes: What do you know?

T1D is an autoimmune disease in which the immune system destroys the pancreas's insulin-producing beta cells, leading to lifelong insulin deficiency and high blood glucose.

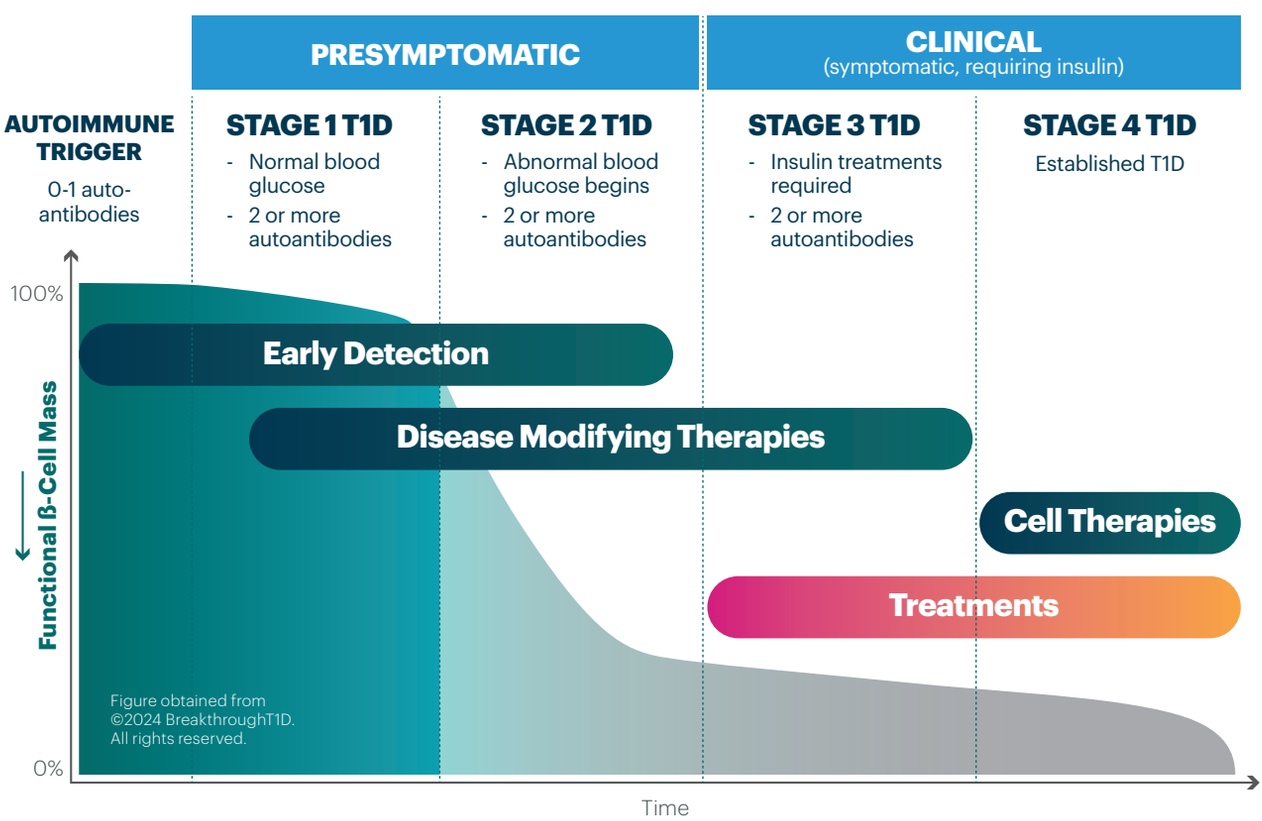
2.7 million

people in the WHO Europe Region are estimated to have T1D.[2]

~31,000 people

develop T1D in the WHO Europe Region every year.

T1D is defined by progressively worsening stages as autoantibodies attack beta cells and blood glucose levels become disrupted.[3]



2. https://diabetesatlas.org/data-by-location/region/europe/
3. Insel RA, Dunne JL, Atkinson MA, Chiang JL, Dabelea D, Gottlieb PA, Greenbaum CJ, Herold KC, Krischer JP, Lernmark Å, Ratner RE, Rewers MJ, Schatz DA, Skyler JS, Sosenko JM, Ziegler AG. Staging presymptomatic type 1 diabetes: a scientific statement of JDRF, the Endocrine Society, and the American Diabetes Association. Diabetes Care. 2015 Oct;38(10):1964-74. doi: 10.2337/dc15-1419. PMID: 26404926; PMCID: PMC5321245.

THE IMPORTANCE OF SCREENING FOR T1D

Clinical diagnosis of T1D typically takes place only at stage 3.

The effectiveness of DMTs in maintaining or even restoring beta-cell capacity has been mainly tested in early stage 3, but may even be more effective in earlier stages.

T1D can be screened through the identification of islet cell antibodies which attack the pancreas. The identification of 2 or more autoantibodies associated with T1D is highly predictive of the future development of stage 3 T1D.

If autoantibodies are detected, a glucose metabolism test can determine the stage of the T1D, based on the degree of disruption of a person's blood glucose level.

T1D SYMPTOMS AND COMPLICATIONS

T1D symptoms include:


Fatigue and Weakness


Extreme Thirst


Increased Appetite


Frequent Urination


Unexplained weight loss


Blurred Vision

People with T1D are at significantly **greater risk of complications:**


Vision loss


Cardiovascular Disease


Kidney Disease


Foot and Lower Limb Loss

Risks of complications can be reduced if type 1 diabetes is detected early and is well managed.

The rising burden of T1D in Europe

T1D is a lifelong autoimmune condition with consequences for quality of life, health risks, healthcare and socioeconomic costs. The condition, which traditionally has been more commonly diagnosed in childhood or adolescence but which also manifests in adulthood, leads to the destruction of the beta cells in the pancreas. This makes the person affected **dependent on external insulin administration for life**.

The condition is associated with **risks of developing many chronic complications**, including cardiovascular diseases, foot loss, vision loss, nerve damage, kidney disease and gum disease.

In addition, **people with T1D must constantly manage their blood-glucose level**, with risks of hypoglycaemia (which may cause seizures and unconsciousness), hyperglycaemia (which may cause complications such as nerve damage and vision loss) and **diabetic ketoacidosis (DKA)**, a potentially life-threatening condition.

An estimated **2.7 million people live with T1D in the WHO Europe Region**.[4] The prevalence of T1D has been rising rapidly in Europe in recent years and is now the highest in the world.[5] A study drawing on data from 32 European countries, including almost all EU countries, found that the per capita incidence of T1D almost doubled between 1994 and 2022.[6]

4. https://diabetesatlas.org/media/uploads/sites/3/2025/04/IDF_Atlas_11th_Edition_2025_EUR_Factsheet.pdf
5. Gong, B., Yang, W., Xing, Y. et al. Global, regional, and national burden of type 1 diabetes in adolescents and young adults. Pediatr Res 97, 568–576 (2025). https://doi.org/10.1038/s41390-024-03107-5
6. Ruiz-Grao MC, Díez-Fernández A, Mesas AE, Martínez-Vizcaíno V, Sequí-Domínguez I, Sebastián-Valles F, Garrido-Miguel M. Trends in the Incidence of Type 1 Diabetes in European Children and Adolescents from 1994 to 2022: A Systematic Review and Meta-Analysis. Pediatr Diabetes. 2024 May 27;2024:2338922. doi: 10.1155/2024/2338922. PMID: 40302967; PMCID: PMC12020782.

The new hope of disease-modifying therapies

The European Medicines Agency (EMA) defines as "disease-modifying" any "treatment that changes the progress of a (long-term) disease".[7] Other definitions have also been proposed, such as "interventions that alter the course of the disease itself".[8] In the context of T1D, this is achieved by preserving or restoring the pancreas' ability to make its own insulin. **DMTs can already slow or prevent the onset of T1D, and could eventually lead to a functional cure.**

This represents a genuine **paradigm shift in the care for T1D**. By addressing the root causes of the disease, **DMTs can potentially free people with T1D from lifelong dependence on external insulin and the risks of many complications**.

DMTs for T1D work by modulating the autoimmune attack against insulin-producing beta cells, making beta cells more resistant to autoimmune attack, or creating and sustaining beta cells. Methods include immune-therapies calming the immune system, gene therapies, cell-based therapies replacing or supporting the individual's cells, and combinations of such therapies. By delaying or potentially halting progression of T1D, DMTs can foster both health preservation and quality of life.

TIME TO PREPARE EU HEALTHCARE SYSTEMS FOR DMTs

EU healthcare systems must be prepared to deploy DMTs if people with T1D are to reap the benefits. Many conditions must be met for this to happen. Among these:

- Programmes and systems must be in place to identify people with T1D among the population.[9]
- Regulators must swiftly and accurately assess the value of DMTs to people with presymptomatic T1D, health systems and society.
- Healthcare infrastructure and qualified professionals must be prepared to administer these treatments.

Detection of T1D at early stages 1 and 2 and education on clinical disease symptoms enables people with T1D and their families to get information about their risk of developing stage 3, reduce the risk of acute and chronic complications, and offer them DMTs.

7. https://www.ema.europa.eu/en/about-us/glossaries/medical-terms-simplifier Other definitions have also been proposed, such as "interventions that alter the course of the disease itself".
8. Mathieu, Chantal. Disease modifying therapies in diabetes and endocrinology. The Lancet Diabetes & Endocrinology, Volume 12, Issue 12, 876 – 877 (2024). https://doi.org/10.1016/S2213-8587(24)00336-X
9. https://www.breakthrought1d.org/news-and-updates/who-is-at-risk-for-type-1-diabetes/

Earlier detection and management of T1D through insulin treatment can prevent complications like DKA and enable children and adolescents to remain in structured and continuous education, successfully complete their academic journey without disruption, and establish a fulfilling and sustainable professional career.[10]

While some promising initiatives are underway, overall **EU healthcare systems remain inadequately prepared to fully leverage the potential of DMTs for T1D**. A coordinated and consensus-driven European strategy should be promoted to ensure that individuals with T1D are identified and, when appropriate and depending on clinical indications and treatment availability, are granted access to the most advanced therapies in the field to delay – and potentially halt – the progression of T1D.

The EU, Member States and healthcare stakeholders should also harmonise practices and ensure equitable access to care for this complex condition, which significantly affects quality of life and presents a high long-term risk of complications. This can ensure more people with early-stage T1D have access to DMTs and people with stage 3 T1D can access insulin treatment to manage the condition and reduce the risk of complications.

Preparing EU healthcare systems for the benefit of people with T1D is possible. This would have **a transformative impact for the lives of tens of thousands of young people and adults** who develop T1D in the EU every year. The following general and specific recommendations outline how to make this happen.

Taking action now is particularly important to **avoid disparities in access** to DMTs as they become available. However, this will depend on preparing healthcare facilities to be able to deliver care, creating care pathways, enhancing healthcare professionals' awareness and having robust screening and monitoring programmes in place to ensure that individuals are uniformly screened, regardless of demographic background, socioeconomic status or ethnicity.

1. Prepare healthcare systems and professionals to deliver DMTs for T1D by implementing workforce training and updating care pathways

European healthcare systems and infrastructure must be prepared to deliver DMTs effectively. Deployment may be hampered by healthcare professionals' limited familiarity with immunotherapies and cell therapies, lack of required staff and integration of DMTs in clinical practice, and the absence of established and widely disseminated protocols for such treatments. These obstacles create uncertainty in the identification and pre-screening of at-risk individuals, infusion scheduling and logistics, and adverse event monitoring and response.

10. Fran Cogen, Henry Rodriguez, Christine A. March, Cynthia E. Muñoz, Jacqueline McManemin, Margaret Pellizzari, Janet Rodriguez, Leah Wyckoff, Alan L. Yatvin, Torie Atkinson, Nuha A. ElSayed, Raveendhara R. Bannuru, Elizabeth J. Pekas, Crystal Woodward, Jennifer Sherman; Diabetes Care in the School Setting: A Statement of the American Diabetes Association. Diabetes Care 27 November 2024; 47 (12): 2050–2061. https://doi.org/10.2337/dci24-0082

SPECIFIC RECOMMENDATIONS TO PREPARE HEALTHCARE SYSTEMS:

- **Ensure healthcare facilities have the capacity** to conduct screening for T1D and administer DMTs.
- **Train healthcare professionals** to diagnose early-stage T1D and administer DMTs. This should include leveraging EU4Health funding to support cross-border training programs for paediatric endocrinologists and nurses.
- **Establish clear pathways for adverse event management, infusion site setup and care coordination**.
- **Establish Centres of Excellence or Reference Sites** for DMTs, where training, infusion protocols, and adverse event management are piloted and scaled. Reference Sites should be chosen according to a set of specifications to ensure only sufficiently competent centres are selected.
- **Promote uniform guidance for more consistent and high-quality care**. This should include developing detailed guidance on the monitoring and treatment of immune-related side effects, which are uncommon in traditional diabetes care.
- **Incorporate protocols** into national diabetes registries and care pathways, aligned with EMA labelling and best practices.
- **Encourage collaboration between national health ministries and patient advocacy groups** to ensure equitable access and reduce regional disparities.

Good practice: The INNODIA network is preparing European clinics for DMTs

The INNODIA project on biomarker discovery in T1D, supported by the EU's Innovative Health Initiative (IHI), has resulted in the creation of the INNODIA network.[11] This pan-European network identifies and supports clinical sites for DMT readiness, both for clinical trials and clinical practice. INNODIA makes Europe a beacon for attracting novel interventions and studies of DMTs, while also bringing DMTs to the people who may benefit from them the moment these reach clinical practice.

11. www.innodia.org

2. Introduce early detection of T1D through islet cell antibody screening as a standard of care

Screening programmes are critical to implementing DMTs for T1D. Screening enables early detection of stage 1 and stage 2 T1D, which in turn can enable monitoring, education and early intervention to potentially change the course of the disease and delay the onset of symptoms and stage 3 diabetes.

The identification of T1D at an early presymptomatic stage has benefits such as giving families time to learn about T1D and avoid emergencies, reducing the risk of developing complications such as DKA, and enabling use of DMTs.

International Classification of Diseases (ICD) codes now exist for early stage T1D. However, **current detection methods for T1D remain inadequate**, limiting the early use of DMTs.

As of 2025, efforts to ensure early diagnosis of T1D, which is crucial to intercept the disease, are patchy across the EU. New programmes should build upon the practices and lessons learned from current screening initiatives, such as Italy's population-wide T1D screening programme and the EU co-funded EDENT1FI project.

The development of T1D screening capabilities and programmes must consider the potential for harm such as anxiety and false positive results, notably by integrating patient perspectives.[12] In general, screening should expand insofar as effective treatments become available.

SPECIFIC RECOMMENDATIONS FOR EARLY DETECTION:

- **Early detection of stage 1 and stage 2 T1D should be progressively implemented into care pathways as evidence and implementation models mature**. General population screening for T1D with an opt-out is an appropriate long-term goal.
- **Expand screening** beyond individuals with a family history to capture the broader population, with clear standards for early detection and intervention, determining who should be targeted, how often and how.
- **Incorporate patient perspectives into screening programmes**, ensuring transparency about the implications of early diagnosis and treatment options. In the absence of a widely accepted care pathway, parents should be made aware of the potential consequences of screening for their lives.
- **Research the psychosocial impact** of screening and early detection of T1D and **ensure psychological support and counselling** for those involved in screening programmes.

12. Chiolero, Arnaud. Low-value population screening. The Lancet, Volume 404, Issue 10456, 935 (2024). https://doi.org/10.1016/S0140-6736(24)01688-X

- **Raise awareness among parents and young people** about the possibilities of screening and treatment during the early stages of the disease. This should notably prompt **volunteer screening**.
- **Eliminate financial barriers to screening**, including lack of reimbursement of auto-antibody testing.
- **Clarify responsibilities for screening**, defining the roles of general practitioners, endocrinologists and other specialists.

Good Practice: The EDENT1FI project to assess 200,000 children and draw lessons

The EDENT1FI initiative[13] aims to assess 200,000 children across Europe to determine whether they have T1D. This is done through blood screening for islet-directed antibodies. The project investigates the possibility of organising general population screening, monitoring and the ethical and health economic impacts. EDENT1FI provides children and their family with continuous support and education to empower them to effectively manage T1D. The psychological impact of early detection of T1D is being actively assessed through input from patients. EDENT1FI is supported by the IHI.

Good Practice: Fr1da

The Fr1da project implemented screening for early detection of T1D in the German states of Bavaria, Saxony, Lower-Saxony and Hamburg. A study found that children diagnosed with presymptomatic T1D during the project had milder diabetes and significantly lower prevalence of DKA at clinical manifestation.[14]

Good Practice: Italy's groundbreaking population-wide T1D screening programme

Italy is pioneering the implementation of population-wide screening for T1D for people aged 1 to 17. T1D has become the most common chronic disease among Italians of this age range. The programme aims to identify presymptomatic cases, delay disease progression and prevent DKA-related complications.[15] France and Belgium have indicated they will soon roll out screening for family members of people diagnosed with T1D.[16] Implementation is being guided by the D1Ce Screen project, which is exploring the feasibility and technical and organisational aspects of nationwide T1D screening.[17]

13. https://www.edent1fi.eu/
14. Hummel S, Carl J, Friedl N, Winkler C, Kick K, Stock J, Reinmüller F, Ramminger C, Schmidt J, Lwowsky D, Braig S, Dunstheimer D, Ermer U, Gerstl EM, Weber L, Nellen-Hellmuth N, Brämswig S, Sindichakis M, Tretter S, Lorrmann A, Bonifacio E, Ziegler AG, Achenbach P; Fr1da Study Group. Children diagnosed with presymptomatic type 1 diabetes through public health screening have milder diabetes at clinical manifestation. Diabetologia. 2023 Sep;66(9):1633-1642. doi: 10.1007/s00125-023-05953-0. Epub 2023 Jun 17. PMID: 37329450; PMCID: PMC10390633.
15. MV. Messina, P. Pozzilli, S. Zampetti, Paediatric screening in Italy as a gateway to secondary prevention in type 1 diabetes, Diabetes Research and Clinical Practice, Volume 224, 2025,
16. https://www.ihi.europa.eu/news-events/newsroom/italy-screens-type-1-diabetes-alongside-edent1fi
17. https://www.iss.it/en/d1ce-screen-copertina

3. Strengthen research and ensure efficient regulatory pathways to support the development, evaluation, and approval of DMTs for T1D

The deployment of novel treatments must be based on sound evidence. Because of DMTs' long-term impact and focus on children, these can be difficult for regulators to assess according to traditional processes. These challenges are not, however, insurmountable.

Investing in research, leveraging digital and cross-border data, and innovative regulatory solutions can enable DMTs to be approved for patients on a sound foundation of evidence.

The more DMTs are developed and approved, the more likely it is different people will be able to access the right treatment for them. The EU should aim to ensure that the right treatment is delivered to the right person at the right time.

SPECIFIC RECOMMENDATIONS:

- **Increase investment in research on T1D** to address existing knowledge gaps and explore novel approaches.
- **Support networks of clinical trial centres**, such as INNODIA, to keep Europe at the forefront of clinical research on T1D.
- **Encourage cross-border collaboration on data sharing and patient registries**.
- Undertake research to **strengthen the evidence base on the long-term effects of immuno-therapies for T1D**.
- **Implement innovative regulatory solutions** to accelerate the review and deployment of DMTs.
- **Gather regulatory decision-makers, payers and clinicians to define relevant value drivers and needed data points** such as biomarkers and evaluation of real-world evidence.
- **Ensure health authorities capture the full value of DMTs** in preserving health, quality of life and productivity.
- **Leverage IHI, EU4Health and Horizon Europe funding** to support digital health tools for care coordination and real-time adverse event reporting.
- **Ensure DMTs are recognised and accessible at national level** after approval by the EMA. This should include collaboration between national health ministries and patient advocacy groups to ensure equitable access and reduce regional disparities.

Contributors

EUDF would like to thank the following experts, as part of our Strategic Forum on Implementation of Disease Modifying Therapies in Type 1 Diabetes, for contributing initial content and/or reviewing and providing feedback on this ***Disease Modifying Therapies for Type 1 Diabetes*** policy paper.

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Notes

Notes

Disease Modifying Therapies

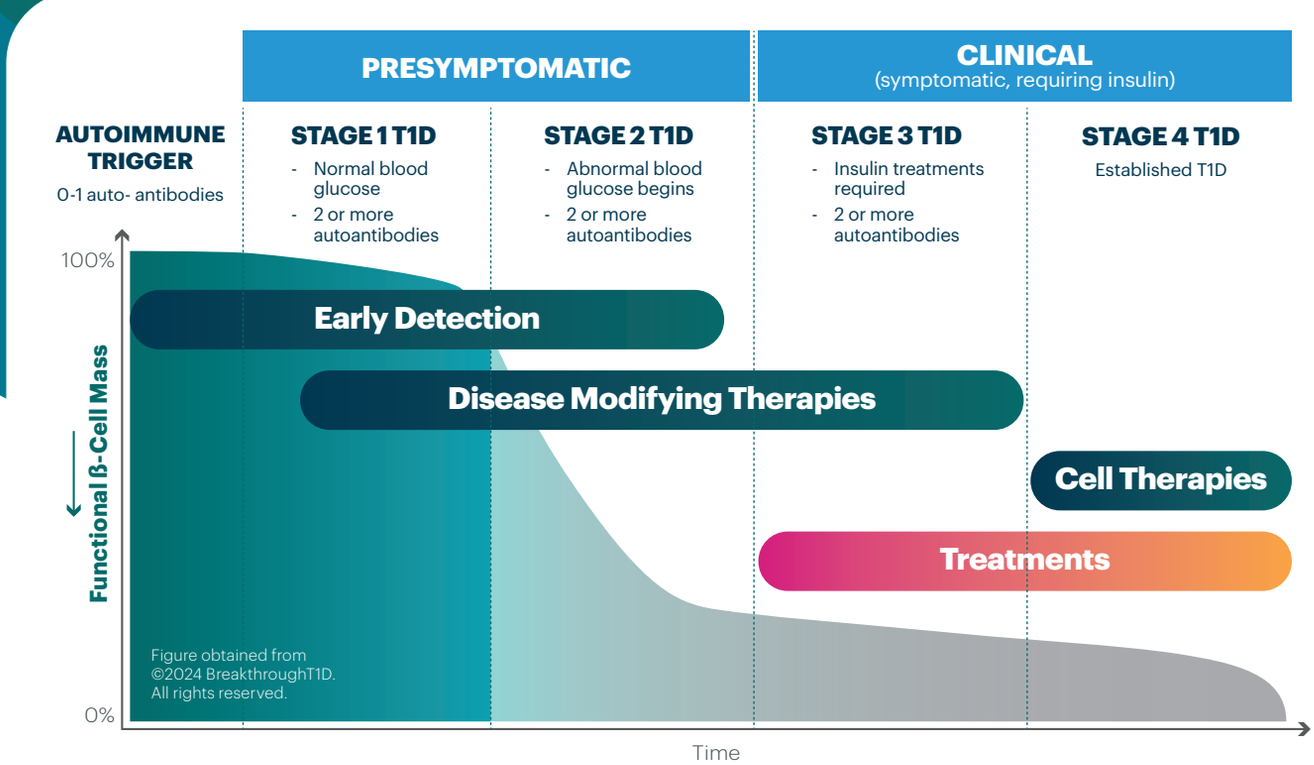
for Type 1 Diabetes

Preparing EU health systems for innovative treatments

Europe's united diabetes community calls for policy actions by the EU, Member States and other healthcare stakeholders to enable the deployment of disease-modifying therapies (DMTs) for type 1 diabetes (T1D) in the coming years.

Recommendations to enable DMTs for T1D:

1. **Prepare** healthcare systems and professionals to deliver DMTs for T1D by implementing workforce training and updating care pathways.
2. **Introduce** early detection of T1D through islet cell antibody screening as a standard of care.
3. **Strengthen** research and ensure efficient regulatory pathways to support the development, evaluation, and approval of DMTs for T1D.



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