Metabolic Support
UK Strategy
2020 - 2030
Welcoming in 2021 comes with a sense of hope that we’ve not experienced before. Waving good-bye to 2020 couldn’t happen quickly enough.

MSUK, like many other charities, have had to make difficult choices as a result of the pandemic. Having to choose the least bad option under difficult circumstances takes its toll, and no one has been immune to the strains of 2020. But the team has worked exceptionally hard and adjusted quickly to serve the on-going needs of our community.

For all the despair and devastation created by COVID-19, there has been many positives. Our digital adoption enabled an increased reach to IMD communities. Despite being physically apart, it feels that we are now closer as a community, with a new sense of unity and togetherness.

Scientific and clinical advances have been staggering. Researchers packed a 10-year process into 12 months. The IMD communities can only dream of the availability of a diagnostic test, effective treatment and preventative vaccinations with regulatory approval and reimbursement within 12 months of identifying the disease. As such, much must be learnt by the response to COVID-19 that we must now explore how to implement widely.

Many ‘wins’ from the pandemic came from international collaboration applied locally, global consensus with local implementation. While the rare disease community has worked globally and collaboratively for years, there has been on-going roadblocks that has hindered progress. Whether it be political will, funding restrictions and constraints, traditional academic demands for publications prohibiting open sharing and obscure financial incentives that make patient advocacy groups, academics and industry compete rather than collaborate. Against a backdrop of economic downturn, we must now address these issues head on, adopting and learning from the COVID-19 response.

Our new 2030 strategy establishes individual support, building communities, empowerment and advocacy for the IMD community in an inter-linked approach. This requires open, transparent collaboration and excellence at every juncture.

Setting a strategy for 2030 in 2020 when our world was turned upside down may seem overly ambitious or out of touch, especially when we can’t be certain of what will be in 2021 even. But it is in stormy weather that a sailor needs a course more so than in calm waters. This strategy will steer us through troubled waters, maintain our course and allow us to emerge strongly at the end of this storm. Terms such as patient centricity, patient engagement and patient empowerment are now not only mainstream but becoming mandatory. The tokenism of patient involvement in grant applications or reimbursement discussions is being replaced with including the patient voice as an equal partner. Our 2030 strategy will ensure that MSUK remain not only current, but in the eye of the storm.

Our future success will be determined by how we respond today. Choosing the least bad option now must also be accompanied with embracing change, adapting quickly, creating new solutions and incorporating digital transformation as a leading collaborator.

Communicating in an information rich but knowledge poor world is difficult. We must plainly state complex issues in a way that the wider public understands, while conveying nuanced qualitative issues to professional stakeholders and collaborators openly.

IMDs, like other rare diseases, have long been considered an ‘orphan’, but as orphaned children do become independent adults, we must now accept both the responsibility and opportunity of adulthood. MSUK will celebrate its 40th birthday in 2021; as we celebrate this major landmark, we also embrace the future, and the new landscape and needs that we must support.

I’m honoured to be part of the journey.

Dr Elin Haf Davies
Chair of Board of Trustees
Our story

Metabolic Support UK is an umbrella patient advocacy organisation founded in 1981, supporting people living with Inherited Metabolic Disorders (IMDs) and their caregivers.

There are approximately 30,000 people in the UK today living with one of over 500 IMDs\(^1\). These are rare, lifelong genetic disorders caused by an enzyme deficiency affecting the metabolic pathway which, if undiagnosed or untreated, can cause irreversible complications, or sadly even death.

Focusing specifically on the rarest of the IMDs, those which have no other patient organisation or support group, Metabolic Support UK delivers a wide range of support and advocacy services to address unmet needs. Using qualitative and quantitative data generated via various methodologies, our small dedicated team works to proactively identify priority needs and develop evidence-based outputs and programmes to ensure the maximum impact for individual patients, collective patient communities and the wider IMD community.

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Our vision for the next 10 years

Metabolic Support UK is an independent, not-for-profit charity committed to working with and advocating for people living with Inherited Metabolic Disorders (IMDs) and their families.

By 2030, we want to see advances across all care and services for the IMD community; this includes early diagnosis, informed and empowered patients, and access to the treatments and services that are available to support families living with these conditions to enjoy a better quality of life.

For all IMDs that are not represented by a disorder-specific patient advocacy organisation, we will provide first line support to people living with IMDs and their caregivers, help to build new communities, and education and support to empower and advocate where needed. Where disorder-specific patient advocacy organisations exist, we will actively signpost to them.
Guiding principles: autonomy, striving for excellence & transparency

Our commitment to the patient community is to make sure that the patient voice is heard across all levels of decision-making and policy implementation.

To achieve this, we follow these guiding principles:

A) Autonomy

Metabolic Support UK is an independent organisation with a governing Board of Trustees overseeing its vision, strategy and monitoring funds, and a team of individuals working hard to deliver the key functions and objectives.

Our partnerships, policies and activities are governed by our ethos of integrity, credibility, democracy and public trust.

B) Striving for Excellence

We are a small but passionate organisation both within the IMD community and the broader rare disease sector, driven and inspired by the commitment and the bravery of those that we support.

We want to:

• Ensure that working and volunteering inspires a sense of purpose and connection to our cause.
• Collaborate closely with other patient groups, and across all relevant partners, to ensure efficiency and best outcomes for patients.
• Share our knowledge and expertise within the relevant decision-making processes and make sure that the value of the patient voice is adequately recognised by all our partners and stakeholders in the IMD world.
• Be an organisation that continuously learns, improves and innovates.

C) Transparency

We owe it to our patients and their families/carers, who have entrusted us with their time, effort, knowledge and funds, to act in an independent and transparent manner. Our work is supported by various pockets of community and corporate funding.

This funding is crucial, ensuring that we achieve the balance between delivering impactful services and programmes to meet our strategic objectives, with the long-term financial sustainability of the charity. Metabolic Support UK will declare all funding sources, either for specific projects or supporting the charity’s core costs, on our website and annual report.

Our strategy

Our long-term strategic pillars have been developed based on our evidence and insight of the needs of those that we support. We commit to working collaboratively and transparently as we strive to achieve our objectives, ensuring the best outcomes for patients and their families.
Pillar 1: Individual Support

Our Goal
We recognise that living with a rare IMD is often emotionally difficult, with higher practical and cost implications. We aim to provide first line emotional and practical support to anyone that needs it via our experienced and qualified support team.

Our Aims
To reach our goal, we have set out the following aims:

1. Accessible information and resources
   - Ensure that disorder information is easily accessible and easy to understand.
   - Develop a resource hub based on trending themes of enquiry and patient needs.

2. Individual and peer support
   - Provide a helpline to patients and families during office hours.
   - Connect patients or carers with a peer or community, where possible, within 2 weeks.

3. Working in collaboration
   - Provide support, advice or signposting to another organisation as relevant within 48 hours.
   - Take a proactive approach to addressing unmet needs based on our enquiry data.

Methodology
We will do this by:

- Offering patient friendly disorder information via our website.
- Providing links to experts and clinicians.
- Providing up to date and accurate information regarding care and referral pathways.
- Providing initial emotional support and then signposting to additional services if needed.
- Signposting to relevant organisations such as partner patient organisations or social care.
- Providing benefits support such as supporting letters and guidance.
- Promoting and developing our Metabolic Connect service.
- Providing information and support regarding travel.
- Ensuring all resources we produce are peer led and reviewed.
Our achievements from October 2019 to October 2020

We are reaching more people living with IMDs than ever before:

- **209** new patients/families contacted Metabolic Support UK for the first time in 2020.
- **43%** new patients / **57%** recurring patients
- We received **1,976** individual support enquiries from patients with **147** different IMDs.

(Metabolic Support UK data, October 2019 – October 2020)
Pillar 2: Building Patient Communities

Our Goal
Provide a means and remove barriers, so patients can come together as a community in their disorder area via online communities, one-to-one introductions or wider peer-to-peer support.

Our Aims
To reach our goal, we have set out the following aims:

1. Communities and Networking
   - Expand communities to include harder to reach cohorts.
   - Develop a network of researchers, scientists, Key Opinion Leaders, and companies for each community.

2. Support and Resources
   - Provide individual support to patients where needed.
   - Develop a disorder/community specific framework per community to address unmet needs.
   - Ensure newly diagnosed patients and families are linked with a disorder specific community soon after diagnosis.

3. Creating Patient Leaders
   - Identify and work with community ambassadors to develop disorder-specific resources to benefit and support members of the community.

Methodology
We will do this by:

- Selecting communities to build via a prioritisation process, based on analysis of our evidence on four key criteria:
  - No existing disorder-specific patient organisation
  - Individual patient needs
  - The disorder is ultra-rare with low patient numbers
  - Therapeutic developments in the pipeline
- Improving our visibility, so patients can find their disorder-specific community.
- Providing different platforms for communities to come together to connect with each other, share their experiences and receive support.
- Undertaking different practices to provide education and support to empower patients and caregivers in each community.
- Delivering ongoing support to existing communities.
- Training and regular contact with community ambassadors, supporting them in their role and empowering them to take the lead.
Our achievements from October 2019 to October 2020

- **29** disorder-specific scoping surveys undertaken, helping us to understand the unmet needs of these small patient populations.
- We have built or regularly engaged with **44** patient communities living with IMDs that do not have a disorder-specific patient organisation, a total of **38,289** members.
- We have built **6** disorder-specific patient community frameworks based on results from scoping surveys.
- We have connected **77** individual patients together via our peer support programme, Metabolic Connect.
- **40** patient-friendly information toolkits created, addressing common areas of need.

*(Metabolic Support UK data, October 2019 – October 2020)*
Pillar 3: Empowerment

Our Goal
We want to ensure that patients of all ages are well informed about their choices and have the means to assert their right to access care or treatment that should be available to them as well as the ability to apply good self-management.

Our Aims
To reach our goal, we’ve set out the following aims:

1. Making Informed Decisions
   - Provide easy access to resources for patients to understand their condition and stay updated about new developments.
   - Ensure that patients are aware of options available ranging from diagnostic tools, treatment options, care pathways to additional support and resources.

2. Opportunities
   - Provide platforms for patients to share the impact of the disorder on their daily lives, share their experiences and empower each other.

3. Enabling Patient Leaders
   - Increase confidence of patients to proactively vocalise their needs and concerns.
   - Empower patients to take lead on their condition and self-care.
   - Contribute to the design and implementation of disorder-specific toolkits and frameworks.

Methodology
We do this by providing:

- Up to date information from relevant researchers, pharmaceutical companies and healthcare organisations.
- Webinars, patient days and meetings.
- Annual conferences and symposiums.
- Metabolic Support UK’s own ambassador scheme, patient insight series and volunteer schemes.
- Awareness of opportunities to become patient advisors on relevant committees, forums and advisory boards.
Our achievements from October 2019 to October 2020

- 30,574 unique users visited our website, a 30% increase on the previous year.
- 49% of page views were of the information in our COVID-19 hub.
- We increased our digital presence, with 10,000 views across 34 different videos and webinars.

(Metabolic Support UK data, October 2019 – October 2020)
Pillar 4: Advocacy

Our Goal
There are many challenges and difficulties faced by those living with a rare metabolic disorder, and we work hard to amplify their voice and make sure it is heard, recognised and equally valued among all players and partners.

Gaining unique perspective from our own patient communities and working with other patient organisations, we can formulate responses and advocate for solutions to mutual issues and challenges faced by patients in areas like research, diagnosis, treatments, services and care.

Our Aims
1. Engaging Communities in Research & Drug Development (R&D) Process
   - Support our communities to meaningfully engage in all stages of care and support.

2. Evidence Generation
   - Generate the data and insight required to ensure that health services, reimbursement agencies and pharmaceutical industries have the necessary understanding to develop services, diagnostics and treatment options that address real need.
   - Create and promote evidence-based publications to share our experience and learnings widely among the extended rare disease community and the public.

3. Collaboration & Campaigning
   - Enable patient engagement, so that the patient voice can be heard and included in policies and service planning.
   - Collaborate with other organisations and stakeholders who share mutual aims to improve the quality of life of people living with IMDs.

Methodology
We will do this by:
- Providing practical support and resources for patients engaging with R&D processes.
- Acquiring the skills, expertise and resources needed to advocate on behalf of individual communities.
- Sharing our learnings and work with other patient organisations and stakeholders aiming for a coordinated effort.
- Using our data and evidence to support lobbying for the welfare of the wider IMD community.
- Seeking opportunities to engage with national and international bodies to raise the profile of IMDs.
For rare diseases, it takes an average of 5 years to reach a diagnosis worldwide for IMDs\(^1\).

According to the ABPI, the research and development of a new drug can take any time from 7-12 years\(^2\).

There are over 300 clinical trials taking place worldwide for IMDs\(^3\).

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https://www.abpi.org.uk/media-centre/blog/2018/august/from-molecule-to-medicine/#:~:text=By%20this%20point%20the%20manufacturing%20starts%20being%20prescribed%20for%20patients.

\(^3\) Metabolic Support UK data, March 2020.
We can’t do this alone

To see advances in care and treatment for all IMD patients over the next ten years, we need the collective voice of the whole IMD community – from patients and caregivers to the medical profession, other patient organisations, industry, researchers, academia, government bodies and beyond. We are calling on you to support our strategy and help us to deliver our aims.

To get involved, please get in touch: contact@metabolicsupportuk.org
Get involved

There are lots of ways to support Metabolic Support UK and help us to continue our vital work!

- Join our community
- Tell your story
- Fundraise for us
- Join us on social media
- Make a donation
- Raise awareness

For more information on ways to support us, visit www.metabolicsupportuk.org

Get in touch
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