

**METABOLIC SUPPORT UK IS A CHARITY REGISTERED IN ENGLAND AND WALES (1089588)
IN SCOTLAND (SC044634) AND A COMPANY LIMITED BY GUARANTEE (04267454).**



METABOLIC SUPPORT UK

**ANNUAL REPORT AND
UNAUDITED FINANCIAL
STATEMENTS FOR THE
YEAR ENDED 31 MARCH
2024**

**METABOLIC
SUPPORT UK**

Your rare condition.
Our common fight.

METABOLIC SUPPORT UK

LEGAL AND ADMINISTRATIVE INFORMATION

TRUSTEES

Miss R E Lindup (Appointed 4 August 2023)

Mr P A Cox (Appointed 4 August 2023)

Mrs M D Williams (Appointed 4 August 2023)

Mr A F Lemoine (Appointed 4 August 2023)

Dr R E Pugh MBChB FRCP

Mr P J Roper MIET CEng BSC Hons Dr R Vara

Mr C Lander

Dr R Sharma (Appointed 18 April 2024)

CHARITY NUMBER (ENGLAND AND WALES)

1089588

CHARITY NUMBER (SCOTLAND)

SC446344

COMPANY NUMBER

04267454

REGISTERED OFFICE

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METABOLIC SUPPORT UK

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**METABOLIC
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Your rare condition.
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TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

THE TRUSTEES PRESENT THEIR ANNUAL REPORT AND FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 MARCH 2024.

The financial statements have been prepared in accordance with the accounting policies set out in note 1 to the financial statements and comply with the charity's governing document, the Companies Act 2006, the Charities and Trustee Investment (Scotland) Act 2005, the Charities Accounts (Scotland) Regulations 2006, FRS 102 "The Financial Reporting Standard applicable in the UK and Republic of Ireland" and the Charities SORP "Accounting and Reporting by Charities: Statement of Recommended Practice applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102)".

OBJECTIVES AND ACTIVITIES: INTRODUCTION TO MSUK

Metabolic Support UK is an umbrella patient organisation founded in 1981, supporting and advocating for people living with Inherited Metabolic Disorders (IMDs) and their communities. We support over 40,000 people living with one of over 500 IMDs in the UK and a large international community. IMDs 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.

Metabolic Support UK delivers a wide range of support and advocacy services to address unmet needs for all IMD communities but with a focus on those for whom there is no established patient organisation. Using qualitative and quantitative data generated via diverse methodologies, our small and dedicated team works to proactively identify priority needs. We develop evidence-based outputs and programmes to ensure the maximum impact for individual patients, collective patient communities and the wider IMD community.

Our vision for the future: By 2030, Metabolic Support UK wants 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.

PUBLIC BENEFIT

The trustees have paid due regard to guidance issued by the Charity Commission in deciding what activities the charity should undertake.

ORGANISATIONAL DEVELOPMENT

This was a year of growth and change for our organisation. We committed to increasing our staff support offering through the recruitment of a new role, Head of Insight and Advocacy, to establish a professional and experienced team member to increase the quality of our internal and external insight provision. This proved immediately effective; we have been praised by both commercial partners and NICE on the quality of our treatment access consultation submissions and our patient advocacy has improved as a result.

TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

We have increased our consultant model, working with external consultants to add value to our work and to ensure our team benefit from expertise of those who work elsewhere in the sector, including a life science consultant and administrative support. The board of MSUK was also refreshed, with us saying goodbye to our Chair, Elin Haf Davies, and introducing a new Co-chair model designed to increase power sharing and lived experience on the board. Our new Co-chairs, Paul Cox and Carl Lander, bring a combination of lived experience, professional expertise of rare disease networks and organisational management.

ACHIEVEMENTS AND PERFORMANCE

This year was pivotal for MSUK; a year in which we deepened our commitment to meaningful patient engagement in the rare disease sector through commissioning pioneering research into quality of life and embedding the learnings from this research into our practice and services. Our key focus always includes hearing directly from our community to inform and support our work. Building on our usual engagement methods to centre our community, our research project Thoughts into Action, aimed to identify what quality of life is as determined by our IMD Community using a research methodology that allowed them to speak on their own terms. Representing over 30 different IMDs, 57 people took part including both adults and children living with IMDs, parents and/or caregivers. The report was launched at an event in Westminster hosted by Liz Truss MP and attended by stakeholders from across the rare disease landscape.



MP LIZ TRUSS HOSTING OUR THOUGHTS INTO ACTION LAUNCH AT WESTMINSTER

Our annual community conference was attended by families from across the UK, healthcare professionals and commercial partners. Sessions included information on clinical trials, clinical research, finances and support and a roundtable on hyperammonaemia.

Our Metabolic Advisory Council (MAC) entered its second year and continues to grow; members have this year contributed their expertise directly into many insight projects and provide in-person representations at scientific meetings on behalf of MSUK. Our phone line rings and emails ping everyday with an increasing number of enquiries from newly diagnosed families and long-term supporters and contacts as we deepen existing relationships and forge new ones.

TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

STRATEGY IMPLEMENTATION

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 with these conditions to enjoy a better quality of life.

Our commitment to our community is to make sure that their voices are heard across all levels of decision-making and policy implementation. We work collaboratively and transparently as we strive to achieve our objectives, ensuring the best outcomes for those affected by IMDs.

In order to achieve this, we developed our long-term strategic pillars based on our evidence and insight of the needs of those we support. These four pillars are:

1. Individual support
2. Building communities
3. Empowerment
4. Advocacy

INDIVIDUAL SUPPORT:

Our individual support focuses on providing those living with IMDs with emotional and practical support tailored to individual need. Offered via, phone, email, social media or video calls, support is provided by our experienced team and may vary between short one-off enquiries or longer-term support. We build vital support networks and act as advocate in a range of fields including education and employment. Each request needs time; we explore the context surrounding each enquiry to allow us to determine what additional support may be required and to ensure that the enquirer is aware of all support on offer. This may include identifying local support for the family, seeking peer support opportunities, or signposting to external organisations offering dedicated disorder-specific support where available.

Our data allows us to see peaks and trends in the themes of enquiries and to develop resources addressing unmet need. Establishing trusted relationships takes time and we facilitate peer support so those with lived experiences can share these with others who may be approaching or considering these themes. Such conversations may be facilitated via our dedicated IMD peer support programme, **Metabolic Connect**.



**HELEN MORRIS,
SUPPORT &
ADVICE
MANAGER**

TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

WHO HAS CONTACTED US AND WHAT SUPPORT DID WE PROVIDE?

In 2023-24 we received a total of 496 enquiries, a 25% increase from the previous year. This dramatic rise in numbers of people reaching out to us for support which can be attributed to improved signposted and accessibility to our information. The majority of these were via email (43%), Website (28%), Phone (20%), Social Media (7%), and meetings, clinics, letter, texts, and video calls making up the remaining 1.8%.

Enquiries received were in relation to 125 different IMDs, with Medium Chain acyl CoA Dehydrogenase Deficiency (MCADD), Hypophosphatasia (HPP), Klippel Feil Syndrome, Ornithine transcarbamylase deficiency (OTC), and Maple syrup urine disease (MSUD) being the most popular. Across the groups, however, our highest number of enquiries fell within the amino acid disorders group which encompasses the urea cycle disorders and organic acid disorders. Our most popular themes of enquiry were peer support, disorder information, treatment and management, and seeking diagnosis – the latter being from those who either did not have a diagnosis but either they or their healthcare provider believed they may have an IMD, this includes those undergoing confirmatory testing for a suspected IMD.

We received 96 international enquires (19% of total) from 38 countries. Most (25%) were seeking information about how to access treatments or potential new therapies (8%), and/or to seek specialist care (10%). Enquirers also wanted to understand more about the treatment and management of their condition (17%) or seek others living with the condition (14%) to reduce a sense of isolation. 30% of international enquiries were from low-and middle-income countries (LMICs) and over half of these enquiries (62%) were relating to accessing treatments or services or seeking clinical trials. We can attribute this to a lack of access to specialist services and knowledge. Public reimbursement for potential new therapies is not common and there is little opportunity to fund access privately and very poor availability of insurance programmes to assist.

To support these families, we used our international connections via memberships such as the SSIEM to gain a better understanding of how we can best support those who have restricted access to healthcare services. In 38% of cases, there were international disorder-specific organisations or rare disorder groups situated within the country who we worked with to offer support and signposting.

INDIVIDUAL SUPPORT ACHIEVEMENTS 2023-2024

INITIATION OF CLINIC VISITS

The return of in-person events post-covid and the return of many of our community attending in-person clinic appointments presented us with the opportunity to expand our face-to-face support. We began discussions and planning with IMD teams to attend metabolic clinics to improve visibility of MSUK, thereby improving access to support. We attended our first general metabolic and MCADD clinics at Great Ormond Street Hospital, London which was a huge success. It allowed metabolic teams to directly signpost families to a person in-clinic to discover more about the support available or to discuss the nature of their clinic visit. Such discussions included transition, being informed of a suspected diagnosis, and travelling abroad.



TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

IMPROVED CONNECTIVITY - IMPROVED SUPPORT

Our community and team benefitted from improved connectivity with our professional network. This included attendance at the BIMDG 2023 annual meeting where we had open discussions around transition programmes and support, paving the way for new resources for families and training support for professionals, we also discussed future clinic visit opportunities, and expanded our contacts to gain a better understanding of IMD services in Scotland. Our clinic attendance provided a unique opportunity to talk candidly with metabolic teams about any gaps in information and support resources and determine areas of work where MSUK could meet this need. We also benefitted from support from clinic teams UK-wide in disseminating our redesigned leaflets and posters for clinic/healthcare settings to improve awareness and signposting and support from adult IMD consultants lending their advice to assist in responding to increasing enquiries around private testing and appointments.

We continued collaborative efforts in the roll-out of the Inherited White Matter Disease (IWMD) Specialised Service and development of the Patient Registry which improves knowledge of this group of conditions and access to care and specialist advice. We spoke at Alex TLCs conference to celebrate the success of collaboration between our organisations and the positive outcomes for our joint community. Similar collaborative efforts included working alongside Genetic Alliance and other patient organisations to drive change for future Managed Access Agreements— sharing our learnings and experiences to improve patient group guidance and community support for future early access programmes and participating in a collaborative effort to improve the provision of genomic information and genetic testing information, an area of growing importance for the IMD community.

INCREASED INTERNATIONAL SUPPORT AND SUPPORT FOR NON-ENGLISH SPEAKING MEMBERS OF OUR COMMUNITY

We also connected with international IMD specialists and other stakeholders to support communities either residing abroad or non-English speaking families in the UK who were struggling to understand their management. This included coordinating a multi-stakeholder approach to assist in accessing medication for an international family during which we expanded our knowledge of different healthcare systems in middle eastern countries. We were able to act on support requests from our clinical networks who were seeking translated information for their patients.

We were able to use our expansive international network to identify specialists to assist in redeveloping or translating advice sheets in Dari and Farsi which allowed the families to clearly understand their child's diet and management plan. Our clinic leaflets were also translated into Punjabi and Urdu. We expanded our connection to international support groups, providing some of these with relevant conversation tools to be used within their own communities and benefitting from these connections to be able to signpost to country-specific or international disorder-specific or rare organisations who were able to assist in the enquiry. We also fully supported an international family to relocate to the UK, assisting in ensuring they had the correct information regarding their visa and health surcharges, assisting in GP registration and urgent referral to a metabolic team for review and treatment, and embedding that family within an online community providing peer support.

TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)



METABOLIC CONNECT REDEVELOPMENT

We reviewed and rejuvenated our peer support programme, Metabolic Connect, improving our sign-up process and developing more substantial terms and conditions to sit alongside a new FAQ document which provides information on the full process from sign-up to connection and beyond. We also formalised our check-in and review process for those taking part in the service ensuring that those joining the service were clear on the support available throughout. We also introduced the addition of webinars and training resources which aims at providing further information about the programme, it's process and progress, as well as addressing helpful topics such as conversation-starters and managing boundaries and self-care. Since the rejuvenation of the project a further 59 people have joined Metabolic Connect.

BUILDING COMMUNITIES

Living with a rare IMD can feel isolating for both the people living with the condition and their families or caregivers. We work to reduce isolation by helping to bring people together to share experiences and ask for advice. Anyone affected by an IMD can join our disorder specific online communities, access one-to-one introductions, or find wider peer-to-peer support. Throughout this support we are keen to remove barriers, creating spaces that are inclusive, safe, and accessible.

In 2023/4 year we focused on learning more about our existing 46 disorder specific groups and their needs, cultivating a growth of over 2600 members across the groups, totalling 41,911 members at the end of March 2024. Taking time to deepen our understanding of our communities affords great learning and engagement opportunities; setting a stable foundation to look to expand the number of groups and communities we support in the coming years.



BRINGING COMMUNITIES TOGETHER

TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

ONLINE ENGAGEMENT

Online communities and spaces are vital for many of our communities and are always a focus of our work. Building on last year's success of increasing the regularity and reach of our social media posts, we focused further on the relevance, brand voice, and value-added elements of our posts. Across all social media platforms (Instagram, Facebook, Twitter/X, and LinkedIn), these actions led to an increased engagement rate including reactions, shares, and comments. We delivered 485 public posts, seen by over 224,000 people, and growing our followers by another 14% to 5,470.

Engaging with others through our own posts, resharing others' posts, and joining conversations consistently has demonstrated that we are reliable and approachable. This has resulted in more community members tagging us, sharing posts in direct messages, signposting to us, and reaching out for support through these platforms.

Our social media channels act as springboards for further engagement and interactions beyond the initial engagement. We launched our Think Ammonia! campaign across our socials and within relevant disorder groups. The campaign received a fantastic reception, being widely shared, and celebrated. Importantly, it was also criticised and instigated important and nuanced conversations that were further addressed in dedicated online sessions bringing the MSUK team and UCD community together for shared learning, deepening our connection and relationships. Each point of engagement provides vital insight and helps us better connect with people.

BUILDING COMMUNITIES ACHIEVEMENTS 2023-2024

- In November 2023 we held our annual community conference attended by over 60 people, bringing together the IMD community for a day of shared learning and vital in-person connection. **"Always nice to connect with others with a lived experience of rare disease in a relaxed setting, but with actual real life tips and knowledge sharing."** - Anonymous Community Member
- We grew both our public, and private, online spaces marking 14% increase in our social media followers and community group members.
- Trialled topic-specific sessions for the fortnightly online Coffee Lounge sessions as part of a continuing review of this long-standing and well-loved support initiative.



**COMMUNITY
CONFERENCE**

EMPOWERMENT

We want to ensure that people living with IMDs are well informed about their choices, at all stages of their life and condition. Our communities should have the means to assert their right to access care or treatment that should be available to them, as well as the ability to provide good self-management.

TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

EMPOWERMENT ACHIEVEMENTS 2023-2024:

1. Metabolic Matters

First launched in January 2022, Metabolic Matters has continued to see growth and development throughout this year. As a platform for sharing our work, the community voice, and the work of organisations we collaborate with, our magazines reached over 1.2 million people worldwide with over 4000 readers. Moving to quarterly productions during this time has allowed for the curation of more in- depth and specialised editions, as well as the introduction of our complementary monthly newsletter, Metabolic Monthly.

2. Metabolic Monthly Newsletter

Introduced in January 2024, our monthly newsletter helps us to share timely updates and opportunities without our mailing list. Featuring recurring segments to empower and connect the IMD community, including our Monthly Medicines roundup, a Metabolic Connect call out, and upcoming Clinic Visits, alongside more temporary opportunities, our newsletter aims to empower and connect the IMD community and its many different stakeholders. Throughout the year our mailing list has grown to 1857 people, including people living with, families, and healthcare professionals, to other patient organisations, charities, and companies, and continues to grow.

3. Monthly Medicines Roundup

Introduced in January 2024, alongside our Metabolic Monthly Newsletter, and featured in our latest news at the start of each month, our monthly medicines roundup provides our community with the latest details on medications for inherited metabolic disorders which are being assessed by the health technology assessment bodies across the UK. The roundup shares which treatments have been approved, but also those that are currently being assessed or have been rejected or suspended.

4. Latest News

Throughout the year, numerous articles have been posted on our latest news section of the website, providing our communities with information on a wide range of topics. This included details about excipients in pharmaceutical products, updates on the UK Government's budgets, Genetic Testing, Benefits and Newborn Screening amongst others. These articles are easy-to-understand and provide details that our communities can utilise to support us in our advocacy activities and to advocate for themselves.

5. Metabolic Clinical Referencing Group (CRG), NHS England

Our CEO, Kirsty Hoyle, represents the voice of our community on the Metabolic CRG, a high profile group of healthcare professionals who are the leading authority on metabolic issues in the UK, providing a direct opportunity to ask questions on behalf of our community.



TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

6. Rare Disease Research Network

Our Head of Insight & Advocacy, Laura Smith van Carroll, was accepted into the management team of the newly established Rare Disease Research Network (RDRN). A project aiming to support the rare disease community in building an online network of partnerships and resources to facilitate new patient-centred research opportunities. As a management team member, she supports the running of the organisation through decision-making, setting goals, reviewing and guiding the overall direction of the organisation. Once launched, the project will enable both MSUK and its community members to actively participate in and lead research related to their disorder.

ADVOCACY

There are many challenges and difficulties faced by those living with a rare metabolic disorder. We work hard to amplify their voices, making sure they are heard, recognised, and equally valued among all stakeholders. There is a lot to learn from our communities and we work to make that happen.

KEY ADVOCACY ACHIEVEMENTS 2023-2024

1. Think Ammonia

The preparation work for the "Think Ammonia!" campaign was undertaken in 2023; this included the launch of the hyperammonaemia survey which received 34 responses from people representing eight individual disorders. The insight from this work was then further bolstered by the conference hyperammonaemia roundtable, and by the expertise of our "Think Ammonia!" working group which comprises eight senior NHS staff including consultants and laboratory representatives



**OUR TRANSLATED
THINK AMMONIA
POSTERS ON
DISPLAY IN A
PERUVIAN
HOSPITAL**

2. Adult Rare Bone Disease Network

This rare disease collaborative network was established to improve care for adults living with rare bone diseases. As the umbrella organisation for six charities supporting people living with these conditions, we were asked to join the secretariat and to provide significant support to the network. As part of this role, we have developed web pages which act as a homepage for the network. This is used by people living with rare conditions and NHS professionals to submit cases for review by experts in the field. We have also undertaken education of our communities about the network and have so far reached 3000 people.

TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)



ADULT RARE BONE DISEASE NETWORK

3. Tyrosinaemia Type One Rollout

Metabolic Support UK are the only patient organisation involved in the working groups tasked with expanding the UK's national screening panel from 9 to 10 conditions. We regularly attend meetings with members of the UK National Screening Committee, the NHS and the UK Health Security Agency. Our role within these meetings is to advocate for our communities to ensure their voice is heard, providing insight on documents, processes and timelines to ensure this rollout is effective and represents what our communities need. We have also engaged our communities and other charities we represent about this work, improving transparency around these complex processes.

4. "Cost of Living with an IMD" report

Our 'Cost of Living with an IMD report' was launched in 2023. This report was well received and reached 454,085 people, generating 880 reads with an average read time of 4 minutes. Further increasing its impact, the findings were referenced in the Specialised Healthcare Alliance report on inequalities for rare diseases which was shared with members of the specialised commissioning team at NHS England (NHSE), those with rare disease portfolios at the Department of Health and Social Care (DHSC) and relevant stakeholders at the National Institute of Health and Care Excellence (NICE)

5. Newborn Screening

We represented our communities on both the IMD Screening Advisory Board and the Newborn Bloodspot Group for Wales. In these groups we participate in regular meetings, sharing our thoughts on various documents and also have the opportunity to raise issues with the heads of screening across the four nations during the "service user" section of the meetings.

6. Signing Open Letters

We have acted as signatories on multiple open letters calling for cheaper energy and water bills for our communities who often face financial issues as a result of their condition. One letter advocated against the closure of ticket offices at train stations and in October (2023), the rail passenger watchdogs officially rejected proposals to close hundreds of train ticket offices across the UK.

7. Mental Health Matters

We released an in-depth article on mental health and IMDs, highlighting the issues that our communities face and the statistics around the inequalities that exist at IMD centres.



TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

8. APPG for Rare and Undiagnosed Conditions

We attended APPG meetings chaired by Liz Twist MP. Here we shared insight from our communities about how policy isn't currently reflecting their needs based on our Thoughts into Action report. We also discussed details about our upcoming "Think Ammonia!" campaign.

9. Representation at national and international conference

Between April 2023 and March 2024, we attended several national and international conferences. Most notable were Society for the Study of Inborn Errors of Metabolism's (SSIEM) annual conference in September 2023, where we hosted the only patient advocacy session in the program "The psychosocial impact of living with an inborn error of metabolism"; the World Orphan Drug Conference (WODC) in November 2023, where we presented the results of our Thoughts into Action research project; and the British Inherited Metabolic Disorder Group in June 2023, where we presented on a panel.

10. Representation on National Forums

We sit on forums and groups that allow us direct opportunities to represent people living with or caring for someone with an inherited metabolic disorder, thereby influencing UK policy, access to treatments and on-going care:

- IMD Newborn Screening Advisory Board (Public Health England)
- Inherited White Matter Disorder Group
- Metabolic Clinical Referencing Group (NHS England)
- Newborn Bloodspot Screening for Wales
- Patient Engagement Group (Genetic Alliance)
- Pyruvate Kinase Deficiency Advocacy and Advisory Council
- Rare Disease Framework Forum (gov.uk)
- Rare Bone Disorder Network
- Tyrosinaemia rollout group
- PNPO Research Project
- X-Linked Hypophosphatasia with Kyowa Kirin
- Lysosomal Storage Disease Collaborative
- Genetic Alliance
- Rare QOL



11. MSUK Insight Services

In 2021/22 we launched our ambitions to deepen and expand our insight services, providing best practice patient insight for all stakeholders. As our previous lead for the MSUK Insight Services left at the beginning of 2023 and our new Head of Insight & Advocacy only started in late June 2023, much of 2023/24 focussed on building an insight pipeline with a few also started at the beginning of 2024. Examples of these projects in 2023/24 include:

HPP landscape review: A survey of the HPP community to determine what improvements they have observed since asotase alfa (Strensiq) was recommended in England and what improvements they consider to still be possible. This will feed into a roundtable discussion by healthcare professionals to gather their views on the same topic. Finally, a summary report with an overview of the successes and challenges will be generated.


TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

Lived experience of nephropathic cystinosis: A survey, originally developed by the Dutch and Flemish cystinosis patient organisation was translated and revised to include UK-specific topics at the beginning of 2024. The survey will be disseminated among people living with nephropathic cystinosis and analysed to draft a report with findings. The report aims to increase the evidence base on the lived experience of the disorder, as well as enable any future health technology assessment responses.

IMD Education Centre: We launched the idea to create an education centre for our IMD community. This saw swift uptake from sponsors, allowing us to scope out three initial e-modules in medicine development, gene therapy and excipients.

HOW THINGS WORK

YOUR IMD EDUCATION CENTRE



OUR IMD EDUCATION CENTRE

MSUK E-Modules

Ever wondered about topics in rare disease and inherited metabolic disorders and thought...I'd like to know more but I don't know where to start? We understand that things can seem overwhelming, so decided to create these easy to understand e-modules to help break down complicated topics so you can have that moment of... "Ahh, I get it now!"

Explore our e-modules by clicking the buttons below to navigate to the course you're interested in:

12. Health Technology Assessments

Involvement in UK health technology assessments (NICE, SMC and AWMMSG) allows us to ensure that our community's voice is heard throughout the entire process of assessing the appropriateness of including a new medication in the NHS. See below for an overview of our health technology assessment work in 2023-2024:

Burosumab (Crysvita), a treatment for children and adolescents (aged 1 to 17 years) living with X-linked hypophosphataemia, was recommended for use by SMC in January 2024. The same treatment for adults living with X-linked hypophosphataemia was still undergoing NICE technology appraisal at the end of March 2024. MSUK supported XLH UK in their initial patient impact data gathering work (including for other health technology assessment bodies) and provided hands-on support during the NICE consultation period in December 2023.

Eladocogene exuparvovec (Upstaza), a gene therapy for the treatment of children 2-years and older living with L-Amino acid decarboxylase (AADC) deficiency, was successfully recommended for use via the NICE highly specialised technologies assessment process in April 2023 and SMC ultra orphan assessment in September 2023. MSUK collaborated with the AADC Trust to develop a patient survey and represent the patient voice throughout the assessments.

TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

Fosdenopterin (Nulibry), a treatment of people living with molybdenum cofactor deficiency (MoCD) type A, was still undergoing NICE highly specialised technology appraisal and SMC ultra orphan assessment at the end of March 2024. Between July and August 2023, MSUK gained the patient perspective through conversations with the community and submitted consultation documents to NICE in addition to attending a NICE scoping workshop in September 2023 where we represented the patient voice and provided details of the impact of MoCD type A. MSUK initiated formal data collection methods to support the patient group submission to NICE and SMC in February 2024, with data collection activities still ongoing as of March 2024.

Mercaptamine bitartrate (Procysbi), a treatment for people living with nephropathic cystinosis was not recommended by SMC in November 2023. MSUK collaborated with Cystinosis Foundation UK and Kidney Research UK to gather patient insight through a survey, case studies and put forward a joint submission.

Pegzilarginase (Loargys), an enzyme replacement therapy for the treatment of people living with arginase- 1 deficiency (ARG1d) was still undergoing NICE highly specialised technology appraisal at the end of March 2024. MSUK initiated formal data collection methods to support the patient group submission to NICE in February 2024, with data collection activities still ongoing as of March 2024.

We also help further clinical trials for IMDs through:

Using Ornithine transcarbamylase (OTC) deficiency as a case study, created a magazine edition to explore different type of treatment currently in development. This helps to inform the wider community about these methods grounded in real-life examples, whilst greatly supporting our OTC community to differentiate and understand the different research and clinical trials available to them.

In order to support the longer-term development of new IMD treatments we contribute to a number of projects that aim to improve access and accelerate development. An example:

Digital Tools for Rare Diseases (DT4RD) project: This project aims to develop a digital platform for assessing upper limb function remotely and continuously. MSUK represent the PPV throughout the project, having developed the patient engagement strategy, patient-reported experience measures and exit interview discussion guide. Additionally, MSUK reviewed patient-facing materials to ensure appropriateness and provided support during the ethical approval process.

FUNDRAISING

Metabolic Support UK's community of fundraisers and donors continued to find creative ways to raise funds. From new to returning fundraisers, their endeavours are invaluable to our work and as always, we are grateful for their hard work and support. We joined EasyFundraising to provide our community with an easy and accessible alternative way for the community to support us.

We were delighted to be successful in our National Lottery bid for a grant of £350,000 over three years; an endorsement of our community work and meeting an aim to diversify our funding as set out in our Thoughts into Action report.

TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

FUNDRAISING 2023-2024:

1. A+ For Effort...

We were delighted that the students at Whickham School chose MSUK as one of two charities to support during their Charity week. Students and faculty came together to hold bake sales, variety shows, competitions, and game shows. Some even skydived!

2. London Marathon 2023

After the 2022 October London Marathon, the 2023 event returned to an April date seeing a drop in our participants from thirteen the previous year to four. We took the opportunity to focus on building a strong rapport with each runner, improving our processes, and using their experiences to build interest for future events. Each runner surpassed their minimum total, raising a combined total of £6,878.

3. Individual Achievements

Throughout the year over 80 people donated or held individual fundraising events on our behalf. From quizzes, sports tournaments, raffles, dances, climbs, and marathons; including an ultra-marathon. Our community came out in force to help us. Thank you.

4. In Memory and Legacies

We are so grateful to those who have left a donation to us in their will, or have raised funds in memory of a loved one. We are here for our community at all stages of their lives, and their families for as long as they need us.



**BECKI
GREENE,
RUNNING
FOR MSUK**

The organisations that provided funding towards Metabolic Support's activities in 2023/24 include:

Alexion, Amicus, Arcturus, Bionical Emas, Cenote, Chiesi, iECURE, Immedica, Jeans for Genes, Kyowa Kirin, Moderna, PTC Bio, Travers, Ultragenyx

TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

FINANCIAL REVIEW

Financial Position at Year End

At the year end, Metabolic Support UK's accounts showed a deficit of £146,406 (2023: £14,628) made up of unrestricted funds.

At the year end, Metabolic Support UK's accounts showed a surplus made up of unrestricted funds. Due to the uncertainty of the impact that the cost of living crisis will have on the charity's income generation in 2023/24 and beyond, the priorities for the next financial year will be to preserve as much of the reserves as possible to ensure the future sustainability of the charity, by:

- Retaining and supporting the smaller existing staff team to deliver the strategy with the support of freelancers
- Building on the success of the National Lottery win to further attract trusts and foundations funding
- Increasing the income stream from paid for services through our insight work

The Board of Trustees of Metabolic Support UK continue to monitor the financial health of the charity.

The unrestricted reserves held by the charity is currently £462,875 - slightly higher than the policy of twelve months running costs (approximately £380,000). However this is a significant decrease from the previous financial year as the board agreed a drawn down of £140,000 from reserves to invest in staff and strategic projects and bring the reserves in line with the policy. This allowed us to build internal capacity to attract new funding streams with a pipeline already secured for the next financial year.

At year ending March 2024, Metabolic Support UK's funds are held in the Co-operative Bank.

The charity's assets are considered to be adequate to fulfil our obligations in relation to future financial commitments. As the majority of our assets are liquid, no significant delays or shortfalls are anticipated in realising these assets into cash.

Reserves policy

It is the policy of the charity that unrestricted funds which have not been designated for a specific use should be maintained at a level equivalent to between three and six month's expenditure. The trustees consider that reserves at this level will ensure that, in the event of a significant drop in funding, they will be able to continue the charity's current activities while consideration is given to ways in which additional funds may be raised. This level of reserves has been maintained throughout the year.

Structure, governance and management

The charity is a company limited by guarantee.

That charity is governed by its Memorandum and Articles of Association and the said Memorandum and Articles of Association were amended in January 2014 and the relevant amendments agreed with the Charity Commission for England and Wales and posted with Companies House.

TRUSTEES' REPORT (INCLUDING DIRECTORS' REPORT) FOR THE YEAR ENDED 31 MARCH 2024 (CONTINUED)

TRUSTEES

The trustees, who are also the directors for the purpose of company law, and who served during the year and up to the date of signature of the financial statements were:

Miss R E Lindup (Appointed 4 August 2023)

Mr P A Cox (Appointed 4 August 2023)

Mrs M D Williams (Appointed 4 August 2023)

Mr A F Lemoine (Appointed 4 August 2023)

Dr R E Pugh MBChB FRCP

Mr P J Roper MIET CEng BSC Hons Dr R Vara

Mr C Lander

Dr R Sharma (Appointed 18 April 2024)

Recruitment and appointment of trustees

New trustees are recruited and appointed by the existing trustees as vacancies arise on the Board.

The trustees' report was approved by the Board of Trustees.

Mr P A Cox
Co-Chair of
trustees



Mr C Lander
Co-Chair of
trustees



29 January 2025



**PAUL COX &
CARL LANDER,
CO-CHAIRS OF
MSUK**

**METABOLIC
SUPPORT UK**

Your **rare** condition.
Our common fight.

INDEPENDENT EXAMINER'S REPORT TO THE TRUSTEES OF METABOLIC SUPPORT UK

FINANCIAL REVIEW

I report to the trustees on my examination of the financial statements of Metabolic Support UK (the charity) for the year ended 31 March 2024.

RESPONSIBILITIES AND BASIS OF REPORT

As the trustees of the charity (and also its directors for the purposes of company law), you are responsible for the preparation of the financial statements in accordance with the requirements of the Companies Act 2006, the Charities and Trustee Investment (Scotland) Act 2005 and the Charities Accounts (Scotland) Regulations 2006. You are satisfied that the financial statements of the charity are not required by charity or company law to be audited and have chosen instead to have an independent examination.

Having satisfied myself that the financial statements of the charity are not required to be audited under Part 16 of the Companies Act 2006 and are eligible for independent examination, I report in respect of my examination of the charity's financial statements carried out under section 44(1)(c) of the Charities and Trustee Investment (Scotland) Act 2005 and section 145 of the Charities Act 2011. In carrying out my examination I have followed the requirements of Regulation 11 of the Charities Accounts (Scotland) Regulations 2006 and the Directions given by the Charity Commission under section 145(5)(b) of the Charities Act 2011.

INDEPENDENT EXAMINER'S STATEMENT

I have completed my examination. I confirm that no matters have come to my attention in connection with the examination giving me cause to believe that in any material respect:

1. Accounting records were not kept in respect of the charity as required by section 44(1)(a) of the Charities and Trustee Investment (Scotland) Act 2005, Regulation 4 of the Charities Accounts (Scotland) Regulations 2006 and section 386 of the Companies Act 2006.
2. The financial statements do not accord with those records; or
3. the financial statements do not comply with the accounting requirements of Regulation 8 of the Charities Accounts (Scotland) Regulations 2006 and the accounting requirements of section 396 of the Companies Act 2006 other than any requirement that the financial statements give a true and fair view, which is not a matter considered as part of an independent examination; or
4. the financial statements have not been prepared in accordance with the methods and principles of the Statement of Recommended Practice for accounting and reporting by charities applicable to charities preparing their financial statements in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102).

INDEPENDENT EXAMINER'S REPORT TO THE TRUSTEES OF METABOLIC SUPPORT UK (CONTINUED)

FINANCIAL REVIEW (CONTINUED)

I have no concerns and have come across no other matters in connection with the examination to which attention should be drawn in this report in order to enable a proper understanding of the financial statements to be reached.

XEINADIN NORTH WEST LIMITED

2 Hilliards Court
Chester Business Park
Chester
Cheshire
CH4 9QP
29 January 2025



xeinadin

STATEMENT OF FINANCIAL ACTIVITIES INCLUDING INCOME AND EXPENDITURE ACCOUNT FOR THE YEAR ENDED 31 MARCH 2024

		Unrestricted funds 2024 £	Unrestricted funds 2023 £	Restricted funds 2023 £	Total 2023 £
	Notes				
Income from:					
Donations and legacies	2	221,519	248,310	-	248,310
Charitable activities	3	-	24,114	-	24,114
Investments	4	6,498	-	-	-
Total income		228,017	272,424	-	272,424
Expenditure on:					
Raising funds	5	2,436	-	-	-
Charitable activities	6	371,987	287,052	-	287,052
Total expenditure		374,423	287,052	-	287,052
Net expenditure		(146,406)	(14,628)	-	(14,628)
Transfers between funds		-	12,504	(12,504)	
Net movement in funds	8	(146,406)	(2,124)	(12,504)	(14,628)
Reconciliation of funds:					
Fund balances at 1 April 2023		609,281	611,405	12,504	623,909
Fund balances at 31 March 2024		462,875	609,281	-	609,281

The statement of financial activities includes all gains and losses recognised in the year. All income and expenditure derive from continuing activities.

BALANCE SHEET AS AT MARCH 2024

	Notes	2024 £	£	2023 £
Current assets Cash at bank and in hand		496,371		621,093
Creditors: amounts falling due within one year	12	(33,496)		(11,812)
Net current assets			462,875	609,281
The funds of the charity				
Unrestricted funds	13		462,875	609,281
			462,875	609,281

The company is entitled to the exemption from the audit requirement contained in section 477 of the Companies Act 2006, for the year ended 31 March 2024.

The directors acknowledge their responsibilities for complying with the requirements of the Companies Act 2006 with respect to accounting records and the preparation of financial statements.

The members have not required the company to obtain an audit of its financial statements for the year in question in accordance with section 476.

These financial statements have been prepared in accordance with the provisions applicable to companies subject to the small companies regime.

The financial statements were approved by the trustees on 29 January 2025

Mr P A Cox
**Co-Chair
of Trustees**

Mr C Lander
**Co-Chair of
Trustees**

Company registration number 04267454 (England and Wales)

**NOTES TO THE FINANCIAL STATEMENTS
FOR THE YEAR ENDED 31 MARCH 2024****1. ACCOUNTING POLICIES****Charity information**

Metabolic Support UK is a private company limited by guarantee incorporated in England and Wales. The registered office is Centurion House, 129 Deansgate, Manchester, M3 3WR

1.1. ACCOUNTING CONVENTION

The financial statements have been prepared in accordance with the charity's governing document, the Companies Act 2006, the Charities and Trustee Investment (Scotland) Act 2005, the Charities Accounts (Scotland) Regulations 2006, FRS 102 "The Financial Reporting Standard applicable in the UK and Republic of Ireland" and the Charities SORP "Accounting and Reporting by Charities: Statement of Recommended Practice applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102)".

The charity is a Public Benefit Entity as defined by FRS 102. The charity has taken advantage of the provisions in the SORP for charities not to prepare a statement of cash flows.

The financial statements are prepared in sterling, which is the functional currency of the charity. Monetary amounts in these financial statements are rounded to the nearest £.

The financial statements have been prepared under the historical cost convention. The principal accounting policies adopted are set out below.

1.2. GOING CONCERN

At the time of approving the financial statements, the trustees have a reasonable expectation that the charity has adequate resources to continue in operational existence for the foreseeable future. Thus the trustees continue to adopt the going concern basis of accounting in preparing the financial statements.

1.3. CHARITABLE FUNDS

Unrestricted funds are available for use at the discretion of the trustees in furtherance of their charitable objectives. Restricted funds are subject to specific conditions by donors or grantors as to how they may be used. The purposes and uses of the restricted funds are set out in the notes to the financial statements.

1.4. INCOME

Income is recognised when the charity is legally entitled to it after any performance conditions have been met, the amounts can be measured reliably, and it is probable that income will be received.

Cash donations are recognised on receipt. Other donations are recognised once the charity has been notified of the donation, unless performance conditions require deferral of the amount. Income tax recoverable in relation to donations received under Gift Aid or deeds of covenant is recognised at the time of the donation.

**NOTES TO THE FINANCIAL STATEMENTS
(CONTINUED) FOR THE YEAR ENDED 31 MARCH 2024****ACCOUNTING POLICIES (CONTINUED)**

Legacies are recognised on receipt or otherwise if the charity has been notified of an impending distribution, the amount is known, and receipt is expected. If the amount is not known, the legacy is treated as a contingent asset.

1.4. EXPENDITURE

Expenditure is recognised once there is a legal or constructive obligation to transfer economic benefit to a third party, it is probable that a transfer of economic benefits will be required in settlement, and the amount of the obligation can be measured reliably. Expenditure is classified by activity. The costs of each activity are made up of the total of direct costs and shared costs, including support costs involved in undertaking each activity. Direct costs attributable to a single activity are allocated directly to that activity. Shared costs which contribute to more than one activity and support costs which are not attributable to a single activity are apportioned between those activities on a basis consistent with the use of resources. Central staff costs are allocated on the basis of time spent, and depreciation charges are allocated on the portion of the asset's use.

1.6. CASH & CASH EQUIVALENTS

Cash and cash equivalents include cash in hand, deposits held at call with banks, other short-term liquid investments with original maturities of three months or less, and bank overdrafts. Bank overdrafts are shown within borrowings in current liabilities.

Basic financial assets

Basic financial assets, which include debtors and cash and bank balances, are initially measured at transaction price including transaction costs and are subsequently carried at amortised cost using the effective interest method unless the arrangement constitutes a financing transaction, where the transaction is measured at the present value of the future receipts discounted at a market rate of interest. Financial assets classified as receivable within one year are not amortised.

Basic financial liabilities

Basic financial liabilities, including creditors and bank loans are initially recognised at transaction price unless the arrangement constitutes a financing transaction, where the debt instrument is measured at the present value of the future payments discounted at a market rate of interest. Financial liabilities classified as payable within one year are not amortised. Debt instruments are subsequently carried at amortised cost, using the effective interest rate method. Trade creditors are obligations to pay for goods or services that have been acquired in the ordinary course of operations from suppliers. Amounts payable are classified as current liabilities if payment is due within one year or less. If not, they are presented as non-current liabilities. Trade creditors are recognised initially at transaction price and subsequently measured at amortised cost using the effective interest method.

Derecognition of financial liabilities

Financial liabilities are derecognised when the charity's contractual obligations expire or are discharged or cancelled.

NOTES TO THE FINANCIAL STATEMENTS (CONTINUED) FOR THE YEAR ENDED 31 MARCH 2024

ACCOUNTING POLICIES (CONTINUED)

1.7. EMPLOYEE BENEFITS

The cost of any unused holiday entitlement is recognised in the period in which the employee's services are received. Termination benefits are recognised immediately as an expense when the charity is demonstrably committed to terminate the employment of an employee or to provide termination benefits.

2 Income from donations and legacies

	Unrestricted funds 2024 £	Unrestricted funds 2023 £
Donations and gifts	221,519	248,310

3 Income from charitable activities

	Unrestricted funds 2024 £	Unrestricted funds 2023 £
Fundraising		
Sale within charitable activities	-	24,114

4 Income from investments

	Unrestricted funds 2024 £	Unrestricted funds 2023 £
Interest receivable	6,498	-

5 Expenditure on raising funds

	Unrestricted funds 2024 £	Unrestricted funds 2023 £
Fundraising and publicity		
Other fundraising costs	2,436	-

NOTES TO THE FINANCIAL STATEMENTS (CONTINUED) FOR THE YEAR ENDED 31 MARCH 2024

6 Expenditure on charitable activities

	Charitable activities 2024 £	Charitable activities 2023 £
Direct costs		
Staff costs	233,121	216,090
Insurance	1,588	1,505
Printing postage and stationery	869	265
Telephone and IT costs	15,247	14,182
Meetings travel and accomodation	14,416	2,934
Sundries	1,433	6,077
Recruitment fees	6,724	9,070
Bank charges	88	99
Projects	80,822	12,976
Marketing	6,200	6,022
Professional fees	3,184	9,075
	<hr/>	<hr/>
	363,692	278,295
Share of support and governance costs (see note 7)		
Support	3,395	5,252
Governance	4,900	3,505
	<hr/>	<hr/>
	371,987	287,052
	<hr/>	<hr/>
Analysis by fund		
Unrestricted funds	371,987	287,052
	<hr/>	<hr/>

7 Support costs allocated to activities

	2024 £	2023 £
Human resource fees	3,395	5,252
Governance costs	4,900	3,505
	<hr/>	<hr/>
	8,295	8,757
	<hr/>	<hr/>
Analysed between:		
Charitable activities	8,295	8,757
	<hr/>	<hr/>
Governance costs comprise:		
	2024 £	2023 £
Audit fees	2,540	2,100
Accountancy fees	2,360	1,405
	<hr/>	<hr/>
	4,900	3,505
	<hr/>	<hr/>

NOTES TO THE FINANCIAL STATEMENTS (CONTINUED) FOR THE YEAR ENDED 31 MARCH 2024

8 Net movement in funds

2024
£

2023
£

The net movement in funds is stated after charging/(crediting):

Fees payable for the independent examination of the charity's financial statements

2,540

2,100

9 Trustees

None of the trustees (or any persons connected with them) received any remuneration or benefits from the charity during the year.

10 Employees

The average monthly number of employees during the year was:

2024
Number

2023
Number

Charitable

4

4

Fundraising

1

1

Administration

1

1

Total

6

6

Employment costs

2024
£

2023
£

Wages and salaries

233,121

216,090

The number of employees whose annual remuneration was more than £60,000 is as follows:

2024
Number

2023
Number

£70,000 to £79,999

1

1

Remuneration of key management personnel

Total remuneration for the key management personnel of the charity was £85,742 (2023: £107,842).

11 Taxation

The charity is exempt from taxation on its activities because all its income is applied for charitable purposes.

NOTES TO THE FINANCIAL STATEMENTS (CONTINUED) FOR THE YEAR ENDED 31 MARCH 2024

12 Creditors: amounts falling due within one year

	2024 £	2023 £
Other taxation and social security	24,885	5,659
Trade creditors Other creditors	210	3,152
Accruals and deferred income	4,551	901
	3,850	2,100
	<u>33,496</u>	<u>11,812</u>

13 Unrestricted funds

The unrestricted funds of the charity comprise the unexpended balances of donations and grants which are not subject to specific conditions by donors and grantors as to how they may be used. These include designated funds which have been set aside out of unrestricted funds by the trustees for specific purposes.

	At 1 April 2023 £	Incoming resources £	Resources expended £	Transfers £	At 31 March 2024 £
General funds	609,281	228,017	(374,423)	-	462,875
	<u>609,281</u>	<u>228,017</u>	<u>(374,423)</u>	<u>-</u>	<u>462,875</u>
Previous year:	At 1 April 2022 £	Incoming resources £	Resources expended £	Transfers £	At 31 March 2023 £
General funds	611,405	272,424	(287,052)	12,504	609,281
	<u>611,405</u>	<u>272,424</u>	<u>(287,052)</u>	<u>12,504</u>	<u>609,281</u>

14 Related party transactions

There were no disclosable related party transactions during the year (2023 - none).

**METABOLIC
SUPPORT UK**

Your rare condition.
Our common fight.