TREAT-NMD Executive Committee
Nominees for Patient Representative 2020

All nominee statements are listed below
Nominees have been listed alphabetically by first name

Name	**Affiliation/Organisation**
Cara Philpott	Muscular Dystrophy WA
Debra Miller	CureDuchenne
Fatima Ielda Oliveira Braga Vaz	Fátima Braga – ABRAME
Joaquim Brites	APN - Associação Portuguesa de Neuromusculares
Joseph EL MIR	Land For Hope, LEBANON / Co-founder and Secretary General
Klair Bayley	Currently General Manager at the Centre for Community-Driven Research (CCDR)
Laura Rufibach	Jain Foundation
Raffaella Willmann	Swiss Foundation for Research on Muscle Diseases and ENMC
Rene Břečťan	PARENT PROJECT Czech Republic
Ryan Fischer (nominated by Ann Martin at PPMD)	Parent Project Muscular Dystrophy
Silke Schlüter	Deutsche Gesellschaft für Muskelkranke e.V. – DGM
SOOK YEE YAP	WE CARE JOURNEY (PERSATUAN KEBAJIKAN CERIAJAYA KUALA LUMPUR DAN SELANGOR (WECAREJOURNEY))
Tara Voogel	Program Manager for Neuromuscular Disease Foundation & Co-Founder for GNE Myopathy International, Steering Committee Member for an NIH Clinical Trial

Nominee One

Name: Cara Philpott

Organisation/Affiliation: Muscular Dystrophy WA

Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date

I have a young son with DMD as a result I have become a passionate patient advocate not only for my son but also for future generations. I sincerely believe there will be an effective treatment, if not a cure, but significant work is required to get there. I spend considerable time reading research, following clinical trials, attending conferences (physical and virtual when possible) and am actively involved within the muscular dystrophy/neuromuscular community in my hometown, Perth, Australia. I am a member of Save our Sons Duchenne Foundation and have held a central role in organising two community walks to raise funds and awareness for DMD/BMD research as well as general fundraising activities. I attend conferences and community meetings with other members. Before joining the Board, I was a member of Muscular Dystrophy WA attending conferences and community events, fundraising activities and networking. Now that I have joined the Board, I am involved in strategy development, governance and driving efforts to achieve our mission and purpose. Through the World Duchenne Organisation, I participated in the Patient Advocacy training program in 2019. This was a fantastic opportunity to learn more about patient advocacy but most importantly it increased my awareness in the differences in availability and access to standardised care, clinical trials and therapy options based on geographical locations. This is an area I wish I could do more. I am involved with a number of community reference groups providing advice on study design from a patient's/career's perspective. These research projects are in conjunction with the Telethon Kids Institute.

Please describe your involvement in TREAT-NMD to date

I have not been involved yet other than to read the newsletters and articles on the website. Regardless of the outcome of my application for the Executive Committee I am keen to explore other ways to be actively involved with TREAT-MND.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee

I have an extensive career in Human Resources and Organisational Development, multiple qualifications and many attributes that make me a great candidate. I hold a Bachelor of Psychology which allows me to understand research methodology and design, ethics, human behaviour, the need for evidence-based results and to interpret scientific papers. My Bachelor of Commerce and Post-graduate Diploma in Management have given me a thorough understanding of business practices and operations. I have a Master of Applied Finance which provided me with advanced financial, statistical and analytical skills to drive business and investing decisions. I also undertook specific training with the Australian Institute of Company Directors to pursue Board and Executive positions where I can influence change. Through my career I a gained a deep understanding of people, culture and organisational frameworks, change management and systems implementations to deliver strategic objectives and governance improvements. Much of my work has focused on strategy development, aligning operations with strategy, vision and values. I have worked with diverse teams across the UK, Europe, USA, United Arab Emirates and other Middle Eastern countries in various industries including mining, oil and gas, engineering, construction and hospitality. I also participate in community reference groups for local research projects focusing on neuromuscular conditions. Recently, I was appointed to the Board of Muscular Dystrophy WA. I am delighted to have an opportunity to serve this organization and the community we support. Lastly, it’s my deep passion, unwavering enthusiasm and love for my family that makes me a great candidate.

Nominee Two

Name: Debra Miller

Organisation/Affiliation: CureDuchenne

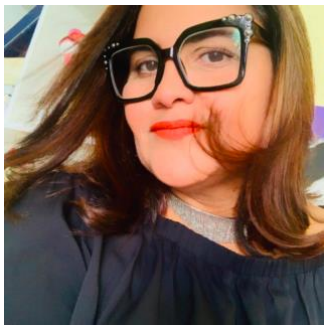
Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date:
Debra has focused on Duchenne Muscular Dystrophy (DMD) for the last 18 years, both as the mother of a 23-year-old son with DMD, and as the CEO & Founder of the patient advocacy organization—CureDuchenne--that she started with her husband soon after her son was diagnosed, providing education to patients and parents, outreach, and advocacy. Debra is integrally involved with families daily, including personally talking with families who may be newly diagnosed or struggling with day-to-day challenges. She interfaces with United States federal agencies including the FDA and various Centers of the NIH to provide insights and help progress drug development. It was the realization that researchers need more access to biospecimens that drove the recent formation of the CureDuchenne BioBank under Debra's leadership. Research on these biospecimens will enable better understanding of the many mutations associated with Duchenne and move us toward precision medicine. Debra's experience with her son's physical therapist grew into a program for physical therapists to provide specific care to Duchenne families and training for physical therapists around the United States and now globally through the online portal. She regularly speaks and attends conferences representing the patient point of view, keeping it front and center while also focusing on supporting research and finding a cure.

Please describe your involvement in TREAT-NMD to date:
Debra has been involved with TREAT-NMD for several years. Through early relationships with academic founders of the TACT Review Committee, Debra was able to ensure early funding from CureDuchenne and nominated Dr. Mike Kelly who became a core committee member. She most recently led the planning to provide funding to TREAT-NMD for academicians to attend TACT reviews. CureDuchenne Biobank is coordinating with TREAT-NMD's effort to ensure harmonized core data sets.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee:
Debra helped to pioneer venture philanthropy, a successful model that de-risks projects at early or critically vulnerable stages and has helped cultivate a more robust DMD drug development pipeline. She has supported innovative research

with both academics and biopharma companies to accelerate science for cures and treatments for DMD. To date, the investments made have led to five successful exits which funded future research investments including seed funding to Exonics which was purchased by Vertex and early funding for Bamboo Therapeutics' gene therapy acquired by Pfizer. Leading the formation of the education and support programs that CureDuchenne brings to the Duchenne community enables her to provide insights on what empowers patients and families combined with a solid understanding of the elements of science and leadership that tend to drive pipeline progress. She can see gaps in care, spaces where collaboration would serve progress, and how her deep and well-networked international relationships may lend themselves to the efforts TREAT-NMD is or will be putting forth. She is a successful fundraiser, having raised over $50M in funds for research and mission dedicated programs. She is passionate about improving the lives of everyone with rare diseases and will continue to work tirelessly for all families with these conditions.

Nominee Three



Name: Fatima Ielda Oliveira Braga Vaz

Organisation/Affiliation: Fátima Braga – ABRAME

Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date

I am the mother of a patient with SMA TYPE I, aged 18. For 15 years, I have been President of the Brazilian Association of Spinal Amyotrophy - ABRAME. In Brazil we are a reference in SMA and other neuromuscular diseases, such as DMD. We actively participate in the implementation of laws for this community.

Please describe your involvement in TREAT-NMD to date

In 2019 I joined as a member of TREAT - DNM. Representatives of patients and family members still from Brazil were not yet actively participating in this select group.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee:

I consider myself an active person for the NMD community in Brazil. We need to have the chance to bring information and treatments to our patients in Brazil who are still so poorly assisted.

Nominee Four

Name: Joaquim Brites

Organisation/Affiliation: APN - Associação Portuguesa de Neuromusculares

Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date:

My connection to neuromuscular diseases came on the day that my son, now 29, was diagnosed with Duchenne Muscular Dystrophy. This happened in 1997. In the same year, I joined APN. The following year, I was asked to join the Board of Directors and, over 22 years and some management positions later, I became President of the organization. It's been an incredible experience. My son graduated in computer engineering, completed a master's degree in "Mobile Computing", and is a programmer at a company linked to moulds for the automotive sector. Today, I have several responsibilities related to rare diseases and neuromuscular diseases. I belong to the Commission for Policies for the Inclusion of People with Disabilities, an organization belonging to the Portuguese Ministry of Social Security. I am the President of the General Assembly of the Portuguese Alliance of Rare Disease Associations and a member of the Installing Committee of the New Representative Organization of Rare Disease Associations. I am also part of the Board of FENDOC - National Federation of Chronic Diseases. In the new era of medicine and gene therapy, my most recent experience, and perhaps the most daring and innovative, was to represent a group of patients for the first time, in Portugal, in discussions about the approval and reimbursement of an orphan drug (Nusinersen), from the national drug regulatory authority (INFARMED). Despite the many promises of treatment for DMD, I have remained vigilant and active in all advances that arise. I actively participate in Advisory Boards of some pharmaceutical companies so that the opinion of those who truly feel the problems of neuromuscular diseases is never lacking.

Please describe your involvement in TREAT-NMD to date:

My connection to the TREAT NMD network has been going on for a few years now. From an early age, almost since the idea of concentrating and treating patient data recording on a single basis, with European roots, appeared that the APN supported the idea. We even financed the first computer system for this purpose. As soon as registration was open, I signed up as an individual member

and registered APN as an Organization. The hope of being able to give more strength to the researchers, who are fighting so hard to discover innovative treatments for neuromuscular diseases, was the light that brought together all the people who, today, are part of this important and respected organization. I believe that, all the effort will be worth it.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee:
I don't know if it is important to evaluate myself. In general, all candidates will be willing to share their experiences, especially those who have more experience in such a delicate subject, because they live it on a daily basis. If not, none of us would have named ourselves. The desire to find the best solution to the many difficulties that represent all neuromuscular diseases, regardless of the age of the patients, to find answers to questions related to care and caregivers, has deeply involved me in this struggle, even though I have a professional life very demanding and active. Strictness, honesty and transparency have always been part of my life.

Nominee Five

Name: Joseph EL MIR

Organisation/Affiliation: Land For Hope, LEBANON / Co-founder and Secretary General

Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date

I am a Doctor of Physiotherapy and I hold a masters of research degree in the same field. I am specialized in the field of respiratory care from the 'Hospice Civil de Lyon', France. My history with NMD patients goes back to 1993. I am currently the head of the respiratory care unit at SESOBEL. I am a lecturer at the Physiotherapy Institute at Saint Joseph University of Beirut. I am actively involved at the university in studies about the NMD care where I supervise undergraduate and graduate research projects. My involvement in the NMD community has been extensive. I have been instrumental in introducing home care ventilation units to the Lebanese community. I am the cofounder and current secretary general of Land for Hope, an Association of Parents of Muscular Dystrophy Patients. I am also the founder and inaugural director of the private Respiratory Care Clinic in Lebanon. The mission of the practice is the diagnosis and the management of respiratory problems of NMD patients. Throughout my career, I have been always providing NMD standard of care awareness sessions for families in numerous Middle Eastern countries. I am a certified MFM trainer and I am active throughout the MENA region. I am a member at large the European Respiratory Society. I've also been invited as a guest speaker to numerous international conferences and congresses held in Europe and the MENA region. I was a participant in translating "The diagnosis and management of DMD, a guide for families" and the MFM to the Arabic language. I have also been on the organizing committee of the first (2013) and the second (2017) Symposium on NMD in Lebanon that were organized by SESOBEL in collaboration with AFM. Currently I am working on the implementation of the SMA and DMD registries in Lebanon.

Please describe your involvement in TREAT-NMD to date

I joined TREAT-NMD several years ago because of my interest in being part of the international neuromuscular community. I have been actively participating in several meetings, master classes, and workshops organized by TREAT-NMD. I

participated in conferences organized by TREAT-NMD or that were in collaboration with other international networks as a speaker or via poster presentations. I have just started to organize a working group that aims at collaborating with TREAT-NMD to set-up the Lebanese registry for SMA and DMD patients.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee

I consider TREAT-NMD an excellent platform for the different stakeholders involved in the follow-up and care of patients with neuromuscular diseases. The organization is important in improving the treatment and the quality of life of neuromuscular disease patients around the world. For these aforementioned reasons, I think that it is important to increase the organization reach and network to countries that are not represented by TREAT-NMD; particularly in Middle Eastern countries where I am involved in. I hope to be part of a collaborative effort that aims at sharing experiences and best management practices that are essential to the improvement of the lives of our patients. I believe I can bring a very unique perspective to the table when it comes to the care of muscular dystrophy patients. I have a long career and experience in the field of NMD. This has enabled me to represent the voice of patients and their families that I've been treating for more than 20 years. Moreover, over the past few years, I have been involved in social responsibility and community projects that raise awareness about NMD. Given my journey, I do feel fully qualified to be part of the Executive Committee at TREAT-NMD specially that: I have a deep knowledge of the particulars of patients with neuromuscular disease, I understand the challenges and needs that clinical teams have in the diagnosis and follow-up of these patients, and I do appreciate the new standards and research outcomes emanating from your esteemed organization.

Nominee Six

Name: Klair Bayley

Organisation/Affiliation: General Manager at the Centre for Community-Driven Research (CCDR)

Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date

I have been involved in the neuromuscular disorders community for almost 13 years, when my son was diagnosed with Duchenne in September 2007. During this time, I have been a patient representative in Australia, raising awareness and advocating for gold standard clinical care, access to services / equipment and funding for research / access to clinical trials for all those living with Duchenne and Becker muscular dystrophy and the wider neuromuscular community. I have worked in both Duchenne Foundation and Save Our Sons Duchenne Foundation (SOSDF) where I was Executive Officer of Clinical Care and Advocacy for 4 years. I developed a registry program which re-launched this year as a 'registry led registry model' to benefit the wider neuromuscular community, the program is called the Australian Neuromuscular Disease Registry (ANMDR) in collaboration with Murdoch Children's Research Institute overseen by Professor Monique Ryan. I developed the nurses program for Duchenne in Australia to improve access to best practice clinical care, and there are currently 5 nurses and also a Telehealth nurse. My professional background as a registered nurse and midwife has been a huge asset to developing these programs, drawing on skills from 8 years as a hospital manager. I have experience in advocacy to government, seeking to improve research funding, awareness of Duchenne and its impact for those living with the condition and their families, to improve access to funding for equipment and access to equitable health services. As a result of understanding the reasons why clinical trials were not coming to Australia I developed the program Duchenne Network Australia (DNA) to improve clinical trial capacity. I am a representative for Duchenne UK's Project Hercules and have a real passion for working collaboratively for the best outcomes of the community not only in Australia, but across the world.

Please describe your involvement in TREAT-NMD to date

I have been a member of TREAT-NMD as an organisation member during my time at Duchenne Foundation and then Save Our Sons Duchenne Foundation. During this time, I have been involved in the registry program collaboration and recently attended the TREAT-NMD Conference in Leiden and the TGDOC / Registry Curators meeting.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee

I think I will be a good candidate as a patient representative, as I have many key attributes that I believe would complement the TREAT-NMD Executive Committee. These attributes include my lived experience having a child and now young adult living with Duchenne muscular dystrophy, these insights not only in the paediatric space but also now during transition and into adult-services has given me experience into key issues that patients and families face every day in accessing best practice standards of care, equipment and access to clinical trial opportunities. My professional skills will also be of value with my nursing, midwifery, health system management and advocacy experience I will be able to provide insightful contributions in areas of developing and implementing strategic plans and providing advice, by drawing on my experiences and those of our family and the wider Australian community. I have skills and experience in public speaking and presenting at meetings and conferences and would be able to represent and promote the work of TREAT-NMD as required. I work well as part of a team, and have experience collaborating internationally which has given me insights in to the challenges facing colleagues around the world. I have advanced critical and analytical skills which will be of benefit, and these alongside my professional experience in clinical areas, research, clinical trials, HTA, advocacy, government engagement, developing strategic plans and all areas relevant to patients and their families / carers I feel make me a good candidate and bringing value to the committee.

Nominee Seven

Name: Laura Rufibach

Organisation/Affiliation: Jain Foundation

Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date

For the past 12 years I have worked for the Jain Foundation, a non-profit disease foundation focused on finding treatments for a rare form of muscular dystrophy called dysferlinopathy (LGMD2B/R2/Miyoshi). I am also a SAC member for the Neuromuscular Disease Foundation. On a daily basis I advocate for the individuals I serve in multiple ways. One of the main advocacy areas I am involved in is helping individuals obtain a definitive diagnosis which is critical for disease management, family planning, and participation in clinical trials. Too often patients aren't receiving a definitive diagnosis because they can't afford the testing and/or their physician doesn't know what testing to perform. Not understanding what is causing their symptoms is an extraordinarily difficult situation for a patient to endure. I have helped develop and manage multiple free diagnostic programs with the JF team (at Emory University) and in collaboration with Sanofi Genzyme and other LGMD foundations that assisted >2,300 individuals obtain molecular testing to aid in the pursuit of a definitive diagnosis. I also oversee programs that provide education and resources to physicians, patients, other advocacy organization, industry, and the FDA. The other area of advocacy I am heavily involved in is clinical trial readiness. This area has many aspects including the education of our patient community about the clinical trial process and the importance of participating in trials, a registry of definitively diagnosed individuals, and identification of the best clinical outcomes. Providing education and resources to all the parties involved in the drug development process helps de-risk the process, makes the disease area more attractive to industry partners, and increases the likelihood of success. My personal drive for accelerating progress for patients with dysferlinopathy is the basis for my career direction and is what motivates me to continue to advocate for patients.

Please describe your involvement in TREAT-NMD to date

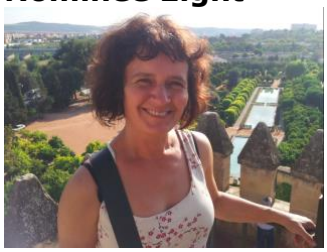
Both the muscular dystrophy foundation that I co-lead (Jain Foundation) and myself have been members of the Treat-NMD since 2017. The Jain Foundation's disease specific registry, the Dysferlin Registry, is listed on the Treat-NMD site and is involved with the TGDOC especially in its endeavours to have all registries

adhere to the same principles and standards, as well as setting up core data sets to allow for better sharing of information across registries. The Jain Foundation has been featured as a spotlight organization in the Treat-NMD newsletter and we regularly submit articles for publication in the Treat-NMD newsletter. The Jain Foundation's Clinical Outcome Study for Dysferlinopathy (COS) is providing patient samples to the Eurobiobank in Newcastle for use by researchers worldwide with the goal of having the validated outcomes measures and care guidelines we are developing be included in Treat-NMD databases so that they are available to all. I am also personally involved with the Treat-NMD in numerous ways. I am the patient representative for the TGDOC LGMD disease area and am also a member of the newly formed Treat-NMD LGMD taskforce and am on the planning committee for the LGMD Masterclasses. I also regularly attend the Treat-NMD conference. I am excited about becoming more broadly involved with Treat-NMD's mission to bring therapies to patients as quickly as possible by becoming a member of the Executive committee.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee

For 12 years I have worked as an advocate of the important role patients play in the development of diagnostic and clinical programs for their disease. Too many drugs have been developed without considering what the "benefit" achieved actually means to the patient. The patients' voices need to be heard at all stages of the drug development process. Through my extensive work with patients with various neuromuscular disorders and my knowledge of the drug development process from target identification and preclinical studies, through patient identification and clinical trials, I believe I can help bring the patient's voice and perspective to all aspects of the process. In addition, I have a PhD in Genetics and have worked on numerous NMDs in academic, industry, and advocacy settings. Throughout my 25 year career, I have investigated the pathophysiology of disease, have developed diagnostic and clinical programs, and have tested possible therapies for various NMDs. I believe my experience and dedication for advocating for the patient voice, as well as my strong scientific training, extensive knowledge of neuromuscular diseases, and experience in academia, industry, and advocacy make me an excellent candidate for the patient representative position. My background uniquely qualifies me to work effectively with all the other members of the committee to reach our common goal of bringing new therapies to NMD patients as quickly and safely as possible. I would be excited and honoured to become part of the Executive Committee. Thank you for your consideration and I would appreciate your vote.

Nominee Eight



Name: Raffaella Willmann

Organisation/Affiliation: Swiss Foundation for Research on Muscle Diseases and ENMC

Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date

As an active member of the ENMC, I have been involved in the efforts of implementing patient involvement in the choices, goals and development of research that addresses neuromuscular conditions, to make sure that advocacy reaches out to the domains typically reserved to scientists and clinicians. We do this by making it mandatory to have the voice of a disease-specific patient or patient representative in every ENMC workshop, with great advantages for both parts. Moreover, in 2018 we organized a special workshop to address the question as to whether patient participation in different research areas (basic, care, trial readiness, registries governance, etc) is implemented at the desired level, and disseminated the outcomes of this workshop by several means.

Please describe your involvement in TREAT-NMD to date

Recent activities: Evaluation, interpretation and publication of 10 years' TACT-impact. Collaboration, publication and follow up work in the TREAT-NMD-Charley's Fund's common project to define the natural history of the D2/mdx mouse model. Past activities: set up and regular update until today of the TREAT-NMD SOPs, application of the SOPs as IRDiRC recognized source, setting up collaborations with CureCMD for the variability analysis of the DyW mouse model, organisation of the 227th ENMC workshop dedicated to improving quality of the preclinical studies, co-organisation of the TACT teaching course at the ICNMD 2018 in Vienna.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee

I am aware that my advocacy experience is not comparable with the one of the present patient representatives. However, with this application I would like to express my motivation to work again for the TREAT-NMD network, which I have supported since the very beginning. I am a proactive, committed and engaged person, with a fairly good network in the neuromuscular community and I am used to address discussions in a constructive and collaborative way. I would be happy to contribute in maintaining the great value TREAT-NMD has in the neuromuscular community.

Nominee Nine

Name: Rene Břečťan

Organisation/Affiliation: PARENT PROJECT Czech Republic

Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date

I am the father of an 11-year-old son with DMD. I have been a member of the Parent Project Czech Republic for 10 years, an organization that brings together patients and parents of children with DMD. I have been working as vice president of Parent Project for the last 9 years. Together with other parents, we have been involved in improving the care of patients with DMD and other neuromuscular diseases. The experience of working with doctors, hospitals, payers, and politicians has also helped me to promote changes in the field of rare diseases in the position of Vice President of Rare Diseases Czech Republic. We actively participate in the creation of a national strategy for rare diseases, legislative proposals, the establishment of centres for rare diseases and their involvement in the ERN. A respected children's neuromuscular centre was established at the University Hospital Motol in Prague thanks to the close cooperation of neuromuscular doctors (mainly Jana Haberlova) with the Parent Project patient organization. The centre received PPMD certification in 2019, as the first in Europe.

Please describe your involvement in TREAT-NMD to date

The PARENT PROJECT has been a member of TREAT-NMD for several years. TREAT-NMD has always been an important source of information for us, especially in the field of care. We translated the materials (Family guide, Diagnosis and management of Duchenne muscular dystrophy, etc.), after their publication in order to get them to our patients and doctors as soon as possible. We also knowledge from TREAT- NMD from the experience of our leading neurological expert.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee

I believe that my experience in the Central European market, where the role of patient organizations is still key to improving the care of patients with rare diseases, can be beneficial for TREAT NMD. I have experience in promoting and creating a care centre for rare diseases (from the patient's point of view),

promoting the reimbursement of medicines for patients with rare diseases, enforcing standards of care (DMD) among patients and doctors. I believe that my strengths include willingness to listen, empathy, resilience and focus on solving problems.

Nominee Ten

Name: Ryan Fischer

Organisation/Affiliation: Parent Project Muscular Dystrophy

Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date

Ryan Fischer currently serves as the Sr. Vice President of Community Engagement for Parent Project Muscular Dystrophy (PPMD) and has been with the organization for 16 years. Within PPMD, Ryan oversees strategic community engagement, patient-preference research, and grassroots advocacy, guiding the patient community on how to interact with members of the US Congress and federal agencies. Ryan also volunteers his time with World Duchenne Organization providing guidance on international community engagement and overall strategy for the organization.

Please describe your involvement in TREAT-NMD to date

Ryan recently became more involved with TREAT-NMD through his work with PPMD's Duchenne Registry. Ryan has participated in efforts focused on developing a core data set for registries as well as presenting at the 2019 curators meeting in Leiden.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee

Ryan would be an excellent person to serve as the patient representative for the TREAT-NMD Executive Committee. Through his community engagement work at PPMD for the past 16 years, Ryan has connected with hundreds of Duchenne and Becker families around the world. He truly understands and respects the patient perspective, and he never stops advocating for Duchenne and Becker families. Ryan's work experience and his dedication to Duchenne, combined with his warm and engaging personality, make him the perfect fit for this role.

Nominee Eleven

Name: Silke Schlüter

Organisation/Affiliation: Deutsche Gesellschaft für Muskelkranke e.V. – DGM

Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date

In 2012 I was diagnosed with myositis. When I wanted to find out about the clinical picture, I had to realize that there is not much information available. Finally, I found the Deutsche Gesellschaft für Muskelkranke e.V. – DGM (German Society for Patients with Neuromuscular Diseases) and became a member there. I decided to join the Diagnosegruppe Myositis of the DGM and became a contact person of the DGM. In this function I lead a local discussion group for patients with various neuromuscular diseases. In 2016 I was elected chairwoman of the Diagnosegruppe Myositis. Together with the members of the working group of this diagnosis group, we hold patient days, prepare information brochures, work together with physician's, scientists, therapists in speech therapy, physiotherapy, occupational therapy, among others and support research. I have been a member of the MYOSITIS NETZ since 2017. I was a member of the steering committee for the foundation of an international myositis association and have been a board member of the International Myositis Society since 2019. I attended GCOM (Global Conference on Myositis) in 2017 and was involved in the planning for the patient workshop at GCOM 2019. I am a patient representative in an IMACS Scientific Interest Group (SIG). As a representative of the DGM I am involved in the current revision of the guideline "Myositis Syndromes" by the Deutsche Gesellschaft für Neurology (German Society of Neurology). Regular meetings are held with representatives of other rare diseases and every year on Rare Disease Day we arrange a local information event for affected and interested people. On the occasion of Rare Disease Day, I was interviewed by a local radio station about myositis.

P**lease describe your involvement in TREAT-NMD to date**

I am a new member of TREAT-NMD. So far I have participated by distributing the patient registries to patients, doctors, therapists and at events.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee

Rare disease patients often find it difficult to obtain information about their disease. The path to diagnosis is often very long and few doctors are well versed in neuromuscular diseases. Research in the field of rare diseases is expensive and rare. Patient registries are urgently needed to collect sufficient data for

research, studies, diagnosis and treatment. As a patient and patient representative, I have been able to gain a good insight into this problem over the last few years. I would like to give patients, family members, therapists an understanding of the diseases and make the daily handling of them easier. In the field of myositis, I have planned the new Patient-Guide-Myositis and managed its development. I would like to support research and the emergence of new registries and act as a representative for the patients' concerns. A very important point is the development of standards in the diagnosis and treatment of neuromuscular diseases. At the IMACS SIG, the MYOSITIS NETZ and the Deutsche Gesellschaft für Neurologie (German Society of Neurology) we are working on standards in the field of myositis. As a patient representative in TREAT-NMD, I would like to get involved in order to support patients accordingly and to represent the interests of TREAT-NMD.

Nominee Twelve

Name: SOOK YEE YAP

Organisation/Affiliation: WE CARE JOURNEY (PERSATUAN KEBAJIKAN CERIAJAYA KUALA LUMPUR DAN SELANGOR (WECAREJOURNEY))

Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date

I co-founded a registered non-profit organisation for Spinal Muscular Atrophy called WeCareJourney (www.wecarejourney.org) in 2016 and help to run this full time. We support hundreds of beneficiaries across a wide range of referral, intervention and support programs (https://wecarejourney.org/about-us#whatwedo). Some of WeCareJourney's programs extend to rare disease communities, some to families with children with disabilities. One segment often overlooked are families with children with a suspected but undiagnosed rare disease, and for these beneficiaries we provide referrals to local and international experts, and symptomatic intervention (e.g. mechanical exsufflation insufflation device program) and support.

Please describe your involvement in TREAT-NMD to date

I was also a 2-term Vice-President of the Malaysian Rare Diseases Society, and current committee member, and have served since 2015. I have presented at global conferences including the World Orphan Drug Congress in USA and in Asia, the Malaysian National Early Childhood Intervention Council conference and Malaysian Paediatric Association conference, and participated at Cure SMA in USA. For many years I have strived to raise the awareness for SMA research, the need to treat, and stakeholder collaboration. Primarily in and for Malaysia and our region, where we face some resource limitations and barriers in awareness, education, research and access to medicines. Access to Spinraza and Zolgensma, the only 2 approved treatments for SMA, is not broadly available in China and the regions of South Asia https://en.wikipedia.org/wiki/South_Asia and ASEAN https://en.wikipedia.org/wiki/ASEAN. Combined though, the nations in these regions make up the big majority of the world's population and patients. With improving (and even in some countries world class) standards of healthcare and strong growth economies (relative to other regions), clinical trials and access remain very challenging. There is a need for greater equity and participation.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee

WeCareJourney, the registered non-profit organisation that I founded and help to run full time, was accepted as an affiliate member of the TREAT-NMD alliance in 2017. Since then, we adopted the Treat-NMD 2007 Standards of Care guidelines for SMA and contributed the local language version for Malaysia https://treat-nmd.org/family-care-guide/malaysian-sma/. This is suitable also for Indonesia. Towards the end of 2019 we launched the first Malaysian (patient reported) SMA registry. It is based fully on Treat-NMD's mandatory registry dataset for SMA. We translated and launched a local language version together with English https://wecarejourney.org/stories/sma-registry.

I am familiar with markets across South Asia and Asia Pacific, where there are unmet needs and opportunities for engagements. I can help expand TREAT-NMD's footprint and raise the profile of our alliance, across various stakeholders in these regions, and also help bridge with USA and Europe where I have some familiarity with. Attributes Foster collaboration across stakeholders and geographies. For example, co-founded the SMA Asia Pacific Alliance which has been successful share knowledge, engage with pharmaceutical partners at senior global levels, to improve equitable access and work together to find long term solutions for reimbursed access sustainable in local health systems. In Malaysia I have organised roundtables of senior doctors and important stakeholders in constructive dialogue and commitment to action plans. Effective advocacy and voice. I speak and appear in numerous press and other media and radio channels. E.g. privilege at the Malaysian Parliament or hosted by (then) Minister of Health

As a mother to a son with Spinal Muscular Atrophy (SMA), I am passionate and committed, working always with clear intentions and values and a structured approach to make a difference in the lives of families with rare disease. I have organised various fundraising events with top corporations in Malaysia, e.g. Maybank

Nominee Thirteen

Name: Tara Voogel

Organisation/Affiliation: Program Manager for Neuromuscular Disease Foundation & Co-Founder for GNE Myopathy International, Steering Committee Member for an NIH Clinical Trial

Please provide a summary of your experience as a patient representative/advocate within the NMD/rare disease community to date

I plan program for rare disease patients globally specifically for GNE Myopathy patients. Some of these programs include: Symposiums, Patient Days, Webinars, Podcasts Patient Monthly Virtual Huddles. I write a Rare Disease Blog whereby I am able to connect with patients globally. I belong to several social media groups such as LGMD group and a private GNEM group. In addition, I regularly attend scientific presentations and symposiums to learn more about rare diseases. I work with patients globally via various mediums to get definitive diagnoses. I have participated as a "subject" in many clinical trial and am currently on a Steering Committee for the NIH. Previously, I worked in a Skilled Nursing Hospital so I was trained in ethics, confidentiality and clinical aspects of assessing patients.

Please describe your involvement in TREAT-NMD to date

I voted for Committee members. I participated in the GNEM-DMP registry and submitted article(s) to the newsletter.

Why do you think you would be a good candidate for the Executive Committee? Please also include key attributes you feel would be of value to the Executive Committee

I am able to connect with patients globally as I speak several languages and because I have been engaging with patients for a more than decade now. I lived and worked in many diverse cultures/countries which helps me to understand patients worldwide. Most of all there are 6 in my family with GNEM and we have had this disease for more than 40 years, so I know the progression stages and I understand the plight of patients. I view my responsibility as a serious dedication to my life.