



# 2020 ANNUAL REPORT

Medicines Development  
for Global Health



## VISION

To be the world's leading  
not-for-profit biopharmaceutical  
company dedicated to the  
development and delivery of  
affordable medicines for the

**people who  
need them most.**



Photo credit: WHO/TDR/Annette C. Kuesel. Used with permission.



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## MISSION

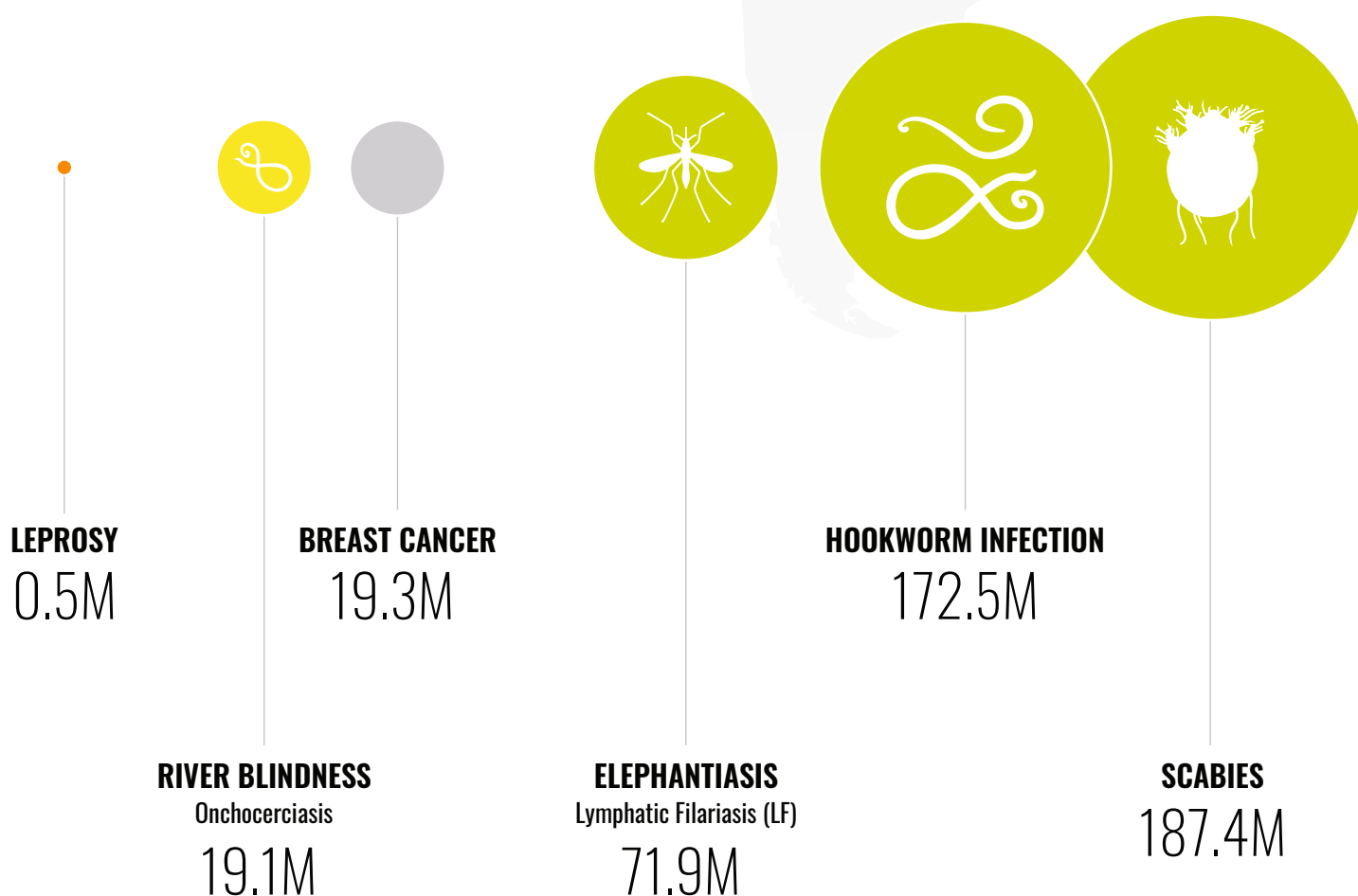
**To address health inequity  
by putting new and improved  
medicines into the hands  
of people who need them most.**



## Prevalence of select Neglected Tropical Diseases and TB, compared with common non-communicable diseases

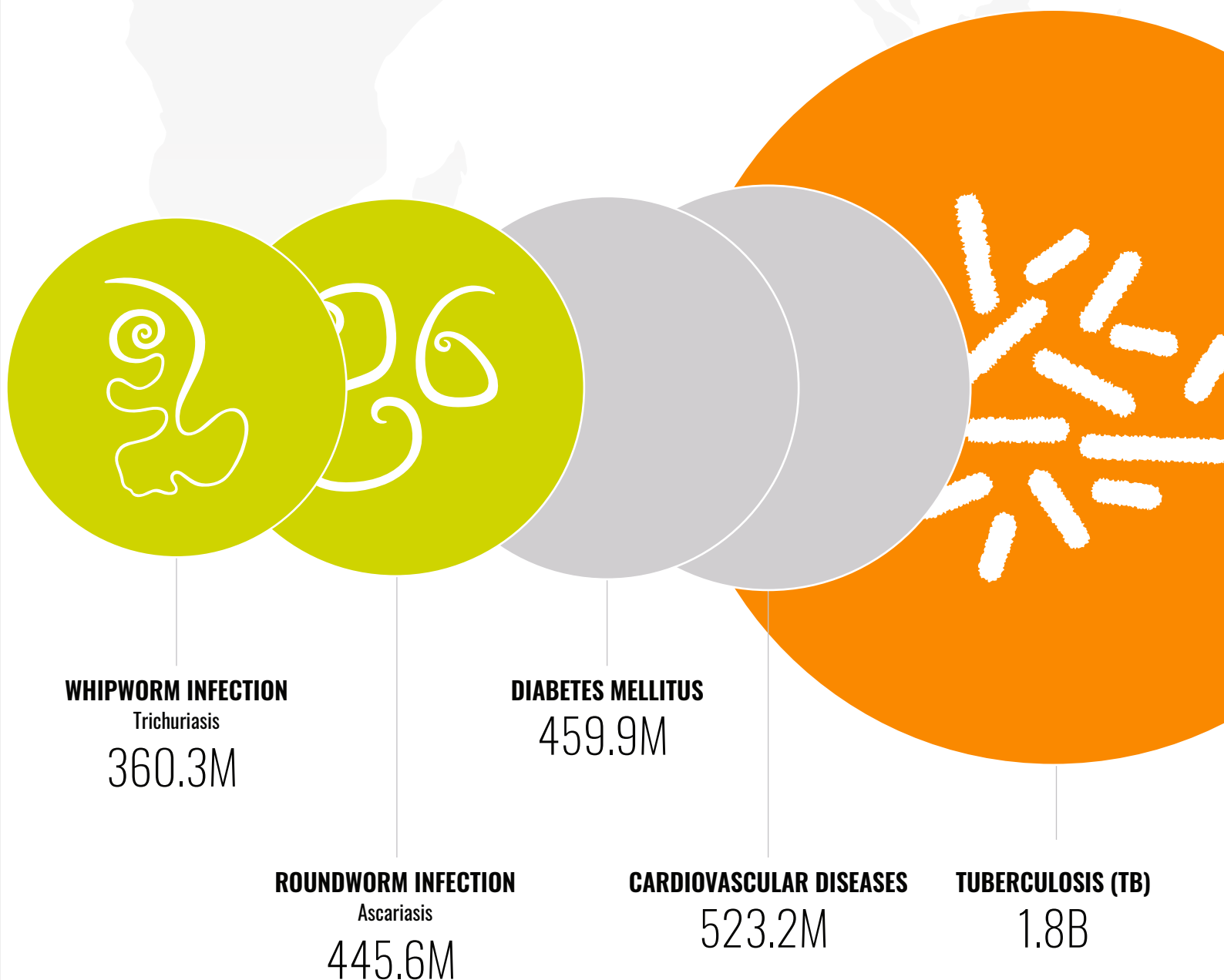
The World Health Organization (WHO) recently released their new 2021-2030 road map for neglected tropical diseases (NTDs), an ambitious manifesto aimed at the elimination of many NTDs. These diseases, primarily endemic in low- and middle-income countries, are devastating. They affect not just the individual, but also the communities and countries where these diseases are most prevalent. However, many people outside of the global public health community are unaware of just how significant many of these diseases are and how difficult it is to get available and affordable medicines to those most in need to treat these diseases.

Data shown is 2019 global prevalence.



Source: Prevalence data adapted from the Global Burden of Disease (GBD) study 2019, IHME

 Common non-communicable diseases



 Moxidectin investigational

 Moxidectin approved

 CC-11050 (AMG 634) investigational



## Letter from Lorna Meldrum, Board Chair

In 2020, Medicines Development for Global Health Limited (MDGH) celebrated its 15th anniversary. When we started on our journey in October 2005, the company had a clear mission that has remained unchanged to today: to address health inequity by putting new and improved medicines into the hands of people who need them most. The company was founded by Mark Sullivan and established as a not-for-profit, registered as a charity with the Australian Charities and Not-for-profits Commission.

Since our founding, MDGH has been expert in the product development process, a rare and much needed skillset, and it has applied those skills to advancing medicines and vaccines that address important unmet medical needs, but which are constrained by having limited market potential. MDGH

*Since our founding, MDGH has been expert in the product development process, a rare and much needed skillset, and it has applied those skills to advancing medicines and vaccines that address important unmet medical needs, but which are constrained by having limited market potential.*

has collaboratively contributed to the evaluation of more than 40 technologies, the majority of which are primarily for diseases affecting people in low- and middle-income countries (LMICs). In 2014, the company became the Sponsor and driver of the development of moxidectin for river blindness

(onchocerciasis) and all other human diseases, when it secured the rights to do so from the Special Programme for Research and Training in Tropical Diseases at the World Health Organization (WHO/TDR).

To fund the completion of the development of moxidectin and the submission of a New Drug Application (NDA) to the United States (US) Food and Drug Administration (FDA), MDGH raised financial support from the Global Health Investment Fund (GHIF), a social impact investment fund structured by JPMorgan Chase & Co. and the Bill & Melinda Gates Foundation. With the invaluable support of WHO/TDR and over 100 experts and specialist companies, the NDA for moxidectin was submitted in 2017. In 2018, the US FDA approved moxidectin for the treatment of river blindness in people 12 years of age and older. Stringent regulatory authority approval is the highest bar in medicine development, and MDGH became the first solo not-for-profit company to achieve FDA approval for a novel medicine.

At the same time, MDGH was also awarded a Tropical Disease Priority Review Voucher (PRV), a US government incentive that rewards innovators who register novel medicines for neglected tropical diseases. MDGH sold its PRV to Novo Nordisk, with the proceeds used to repay investors and support MDGH's ongoing development of moxidectin. As part of their investment, GHIF agreed to form a co-owned entity with MDGH (Atticus Medical Pty Ltd), to provide MDGH with ongoing financial support.

Most recently, we secured from Amgen the exclusive worldwide rights to CC-11050 (also known as AMG 634), an investigational treatment that holds promise in tackling two of the world's most challenging diseases: tuberculosis and erythema nodosum leprosum, an inflammatory complication of leprosy (which is also known as Hansen's Disease).<sup>1</sup>

Even after 15 years, it is still early days for MDGH. We have much more work to do with moxidectin. We have a new compound in our portfolio, and we are bringing on more talent to help us achieve our goal of delivering these medicines to those who need them most.

All of our efforts to date could not have happened without the help and generous support of colleagues, collaborators and contributors. We are grateful to all those who have offered their sage advice, their partnerships and their funding to support our work in our first 15 years.

We hope you enjoy reading about what we have done at MDGH and what we will be doing in 2021 and beyond.



**Lorna Meldrum, PhD**

*Chair of the Board, Medicines Development for Global Health*



Photo credit: WHO/TDR/Annette C. Kuesel. Used with permission.



## Letter from Mark Sullivan, Founder and Managing Director

Thank you for your interest in MDGH. While we are proud of our achievements to date, our “overnight success” has been a long time coming and we have only just begun.

For any biopharmaceutical company, an FDA approval is the height of accomplishment—it represents achievement of the highest standards across the technical, scientific and operational complexities that it takes to generate a new medicine. That the development of a new medicine is a technically challenging and expensive endeavor, with no guarantee of success, is not news. According to Evaluate Pharma<sup>2</sup>, approximately US\$179 billion is currently being spent on pharmaceutical R&D every year and this buys roughly 40 novel medicines annually. For compounds exiting the research arena and entering development, the chances of successful registration are frighteningly low: according to Bio, less than 10% of medicines entering Phase 1 clinical trials ever make it to licensure.<sup>3</sup>

For those working in neglected tropical diseases, the odds are even more daunting. Apart from the additional requirements of the World Health Organization (WHO) before widespread distribution is possible, you are often working where there is limited medical infrastructure (which, in extreme cases, has to be built de novo), and with very limited funds. Humanity’s success in achieving better

*We designed MDGH to be lean, sustainable, innovative, entrepreneurial, data-driven, impactful and game-changing.*

medicines for these diseases is telling: in the context of approximately 400 New Drug Applications (NDAs) approved in the last 12 years, just 12 were for neglected tropical diseases and, before moxidectin, only one was a new chemical entity not already approved for use outside of the United States.<sup>4</sup> One of the key reasons that neglected diseases are neglected is because there is little hope for return on investment, leaving

philanthropy as the primary means of funding development cost and risk. Improving peoples’ lives is the goal as, arguably, the most significant advances in disease management result from the arrival of a new medicine. Due to new medicines, hepatitis C is a curable disease, and HIV/AIDS is a disease people can live with rather than die from. Developing new and improved treatments is something we simply have to find a way to do.

MDGH’s approach has been to operate as a social enterprise, bringing a new model to the field of global health. We designed MDGH to be lean, sustainable, innovative, entrepreneurial, data-driven, impactful and game-changing. Nowhere is the value of this model better exemplified than in our moxidectin development program. The moxidectin development program was started by WHO/TDR in the late 1990s in collaboration with Wyeth and, despite WHO/TDR’s heroic efforts to complete the development



Photo credit: WHO/TDR/Annette C. Kuesel. Used with permission.

program over nearly 20 years, moxidectin was stalled and at risk of becoming a lost opportunity for river blindness sufferers and the global health community. An extraordinary global health medicine would have been lost to the world. The relationship with WHO/TDR and moxidectin itself are exemplars of what is possible in developing and delivering medicines to those most in need. Our newest opportunity with CC-11050 (AMG 634), licensed from Amgen, for erythema nodosum leprosum (a complication of leprosy) and tuberculosis, provides further validation of this model.

We have already demonstrated that a small but passionate, entrepreneurial not-for-profit organisation can marshal the talents, external resources, collaborators and financing required to develop a much-needed new treatment for one of the most challenging neglected tropical diseases.

The potential for MDGH to be game-changing is substantial. We have shown what is possible with our streamlined virtual project team, which is scalable and international. As evidence of the effectiveness of our model, MDGH became the first not-for-profit to achieve solo US FDA approval; the first not-for-profit to receive (and sell) a Priority Review Voucher (PRV); the first not-for-profit to raise capital on the basis of a potential PRV sale; and the first to show that proceeds of the sale of our PRV will remain committed to global health projects – funding the supply and further development of medicines for neglected diseases.

Finally, as an independent company, we are proud to set our own direction and to take on any project of impact, regardless of therapeutic area. We are not reliant on a particular funding source or the priorities of any one funding body.

We see the neglected disease landscape and, for that matter, the overall healthcare landscape for LMICs as being in a state of change. We know we are but one piece in the giant puzzle of factors that can redress health inequities, but we intend on growing and being a significant contributor to helping solve that puzzle.

Please read through our first Annual Report, to see what we have accomplished and where we plan to go. We encourage you to join us on our journey. From whatever part of the global health community you come from, we want to listen to what your challenges are, and we invite you to get to know us better. It is only through such dialogue that we can all collaborate to help resolve the health inequities that persist around the world today. It has been a long journey from our beginnings, working on HIV/AIDS therapeutic and prophylactic vaccine development, but our mission and model of operation has not changed.

Mark Sullivan

**Mark Sullivan**

*Managing Director, Medicines Development for Global Health*



Photo credit: WHO/TDR/Annette C. Kuesel. Used with permission.

## Pharma Research & Development Spend

2018 R&D SPEND (US\$)



**4.06B** Neglected diseases\*  
**179B** Other disease areas

PREVALENCE (CASES)

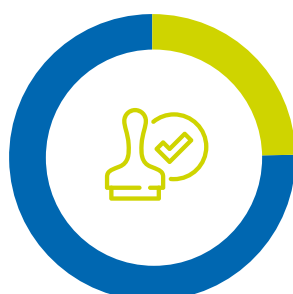


**3.5 billion** NTDs, TB, malaria and HIV/AIDS  
**3.9 billion** Other disease areas

Source: G-FINDER 2019. \*Includes investment in NTDs, TB, malaria and HIV/AIDS priorities in LMICs; EvaluatePharma. World Preview 2019, Outlook to 2024; Global Burden of Disease study 2019. IHME.

## The Paucity of New Treatment Options

FDA APPROVALS OVER THE LAST 10 YEARS (n=1261)



**947** Non-original  
**314** New agents

**INFECTIOUS DISEASES:** 48 new agents  
**NEGLECTED TROPICAL DISEASES:** 12 agents

Source: Center for Drug Evaluation and Research (CDER), US FDA

## MDGH DRUG DEVELOPMENT MODEL

# What makes us different?


There is a fundamental inequity in the development of and access to medicines. Of the approximately 7.7 billion people in the world, 6.4 billion are living in low- and middle-income countries (LMICs) where innovative medicines are rarely accessible. The majority of new medicines are developed for diseases that are prevalent in high-income countries as the potential returns on an approved medicine offset the high cost and risk of development. This model is particularly punitive for the world's poorest populations—according to the World Health Organization, an estimated two billion people do not have access to even the most basic of essential medicines.<sup>5</sup>

MDGH was established with a vision to address important and unmet medical needs in global health through the development and delivery of new medicines as opposed to basic or discovery research.

*The MDGH development model is built on identifying and integrating the necessary expertise from each part of the development pathway at the time that expertise is needed.*

Product Development Partnerships (PDPs) remain a role model for MDGH, bringing their skill set to partnership-based product development with (in the majority of cases) the biopharmaceutical industry. As documented in a recently released report, *Keeping the Promise*, co-authored by MDGH, the PDP model has demonstrated what is possible in global health.<sup>6</sup> We saw an opportunity to create

something that combined the best of the two models – of PDPs and traditional biopharma – into an entity that would enable us to focus on developing and delivering medicines to those most in need while maintaining self-determination as a not-for-profit company.

The MDGH development model is resource-efficient and cost-effective, proven through the moxidectin NDA and is intended to reduce the time and expense of bringing medicines through development. It is built on identifying and integrating the necessary expertise from each part of the development pathway at the time that expertise is needed. 



Women of the Dagon ethnic group carrying baskets of harvested millet back to their village - Mali  
 Photo credit: Phil Marion for Getty Images

# The Market Failure

## ACCESS TO MEDICINES



Source: The World Bank, data.worldbank.org | Photo credit: TB Alliance. Used with permission.

## MDGH Current and Near-Term Portfolio

COMPOUND	INDICATION	PRECLINICAL	Phase 1	Phase 2	Phase 3	NDA	3B/4	WHO	
								STAG	Guideline inclusion
Moxidectin	Onchocerciasis	Paediatric, multiyear, WHO guideline, MDA programs							
	Scabies	Phase 2a dose finding							
	Soil-Transmitted Helminths	Proof of concept complete							
	Lymphatic filariasis	Study initiated Q2 2020							
	Strongyloidiasis	Long-term follow-up ongoing							
	Head lice								
CC-11050 (AMG 634)	Leprosy	Proof of concept complete							
	Tuberculosis	Proof of concept complete							

**Note:** Development of moxidectin for head lice is being considered but has not yet been initiated.

MDGH is developing two medicines: moxidectin and CC-11050 (also known as AMG 634). Together, moxidectin and CC-11050 (AMG 634) are targeting seven different diseases that collectively affect approximately three billion people.<sup>\*7-9</sup>

Moxidectin is a macrocyclic lactone and is from the same family as ivermectin, which is one of the most highly regarded medicines in the world today. Moxidectin is highly active against a broad variety of parasitic worms and ectoparasites and this effect has been exploited for decades in veterinary medicine.

CC-11050 (AMG 634) is a phosphodiesterase type 4 (PDE4) inhibitor from the same class of medicines as Otezla®, a well-known treatment for psoriasis, marketed by Amgen. CC-11050 (AMG 634) modifies the host's immune response to infection and is being studied for the treatment of tuberculosis and erythema nodosum leprosum. 🟡➕

<sup>\*</sup>According to the IHME Global Burden of Disease study, these diseases (onchocerciasis, scabies, soil-transmitted helminthiasis including strongyloidiasis, lymphatic filariasis, leprosy and tuberculosis) collectively caused 57 million Disability Adjusted Life Years (DALYs) and had a prevalence of 3.0 billion cases in 2019. Tuberculosis accounted for the vast majority of the burden, with 47 million DALYs and a global prevalence of 1.8 billion cases in 2019.<sup>7</sup>

# Moxidectin to accelerate the elimination of river blindness

Onchocerciasis (river blindness) is a neglected tropical disease caused by the parasitic worm, *Onchocerca volvulus*, which is transmitted to humans via repeated bites from infected black flies. The resulting infection can lead to severe debilitating and disfiguring dermatologic manifestations, visual impairment and even blindness. More than 200 million people are at risk for these infections and almost all infected people live in 31 African countries.<sup>9</sup> Ivermectin is the current standard of care for treating river blindness and has been distributed to areas that are endemic for the disease for over 30 years. However, there are still many areas where the prevalence of river blindness remains high, even with annual community-directed treatment with ivermectin. Some barriers to effective treatment cannot be addressed, such as conflicts and geographical accessibility, but in some areas, suboptimal response to ivermectin is a key issue.

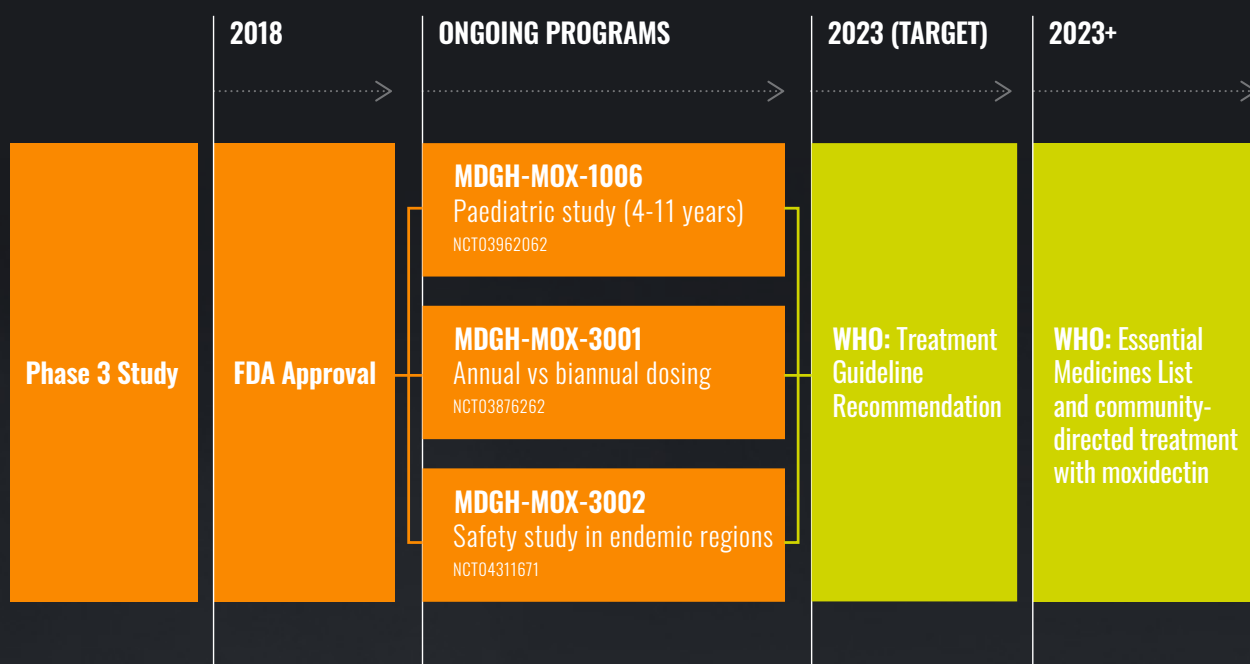
In 2018, MDGH received US FDA approval for moxidectin for river blindness for people aged 12 years and older. MDGH is now generating the additional data required to implement community-directed treatment with moxidectin in endemic areas that need it, through targeting its inclusion in World Health Organization (WHO) river blindness treatment guidelines. Three clinical studies form the basis of these activities:

a multi-dose trial comparing annual versus biannual dosing of both ivermectin and moxidectin (MDGH-MOX-3001, NCT03876262; Democratic Republic of Congo, DRC), a single-dose safety study of moxidectin in over 12,000 participants (MDGH-MOX-3002, NCT04311671; DRC), and a study in children four years of age and older (MDGH-MOX-1006, NCT03962062; Ghana).



In addition to these clinical trials, a cutting edge comprehensive pharmacokinetic modelling program is being undertaken to better understand moxidectin's potential use in breast feeding and pregnant women. Further disease modelling is also being undertaken to determine how moxidectin could reduce time to elimination of this disease. Additionally, a Phase 2 clinical trial is planned in areas with *Loa loa* co-endemicity to assess safety in this common co-infection, as well as paediatric formulation studies to determine the best format for use in children under 4 years of age and others unable to swallow tablets. Additional implementation work being undertaken includes seeking local regulatory approval in African countries and evaluation of pilot implementation studies in several river blindness endemic countries. Finally, we are continuing to explore the most effective ways of manufacturing and distributing moxidectin. 🌱+

# Onchocerciasis trials: Critical path to access



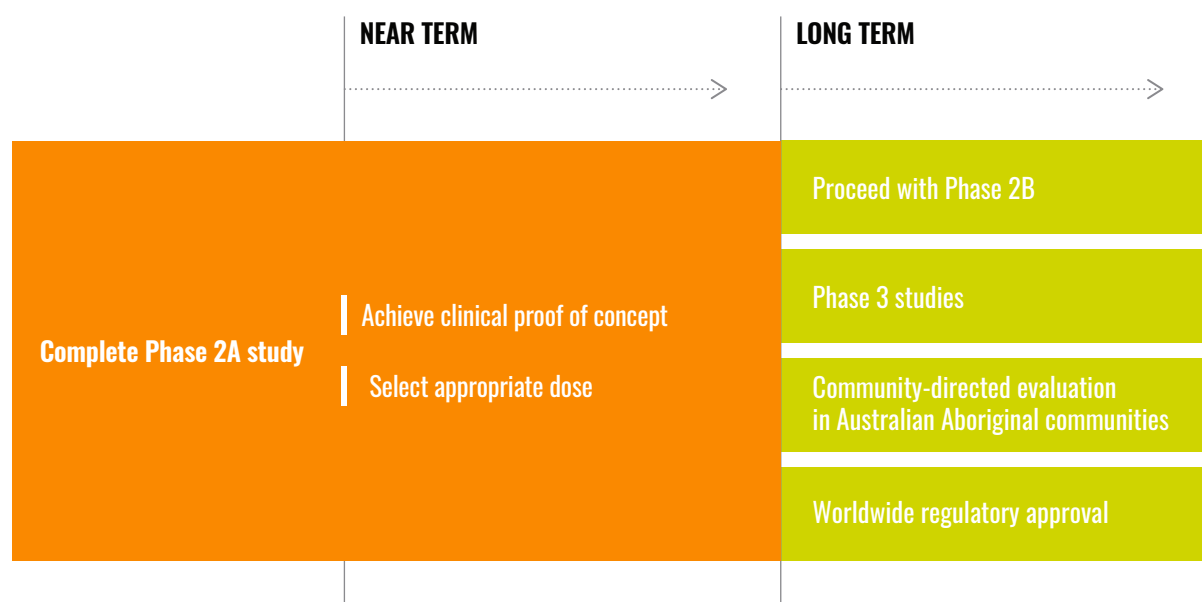
*Planning for sustainable manufacture  
to allow community-directed treatment  
with moxidectin*

# Development of moxidectin for scabies

Scabies, caused by the *Sarcoptes scabiei* var. *hominis* mite, is one of the most common infectious dermatologic conditions and accounts for a substantial proportion of skin diseases around the world, particularly in LMICs and commonly in children. The WHO estimates that, at any one time, more than 200 million people suffer from scabies.<sup>9</sup> The disease occurs worldwide and can affect anyone, although the highest rates of infestation are seen in hot, tropical climates. The burden of disease is particularly high in India, the Pacific Islands and in Australia among Aboriginal communities. In 2017, scabies was included in the WHO list of neglected tropical diseases.

Complications from scabies infestations can lead to secondary bacterial skin infections that compound the burden of disease by increasing the risk of nephritis, rheumatic fever and sepsis, especially in LMICs. The current treatments for scabies include first-line topical agents (permethrin and benzyl benzoate) and oral ivermectin, usually reserved for more serious cases. The biggest drawback to using topical agents is their need to cover and remain on the entire body for between 8-24 hours, depending on the agent and the severity of the infestation. The challenges with ivermectin therapy are that it is approved for use in only a handful of countries and thus is not accessible to the vast majority of people in need. In addition, ivermectin has a short half-life, which usually results in the need for a second dose to be administered to ensure mites from hatching eggs are eliminated. Clearly, there is a need for a better, longer-lasting oral treatment option.

MDGH initiated a Phase 2a dose-finding study (MDGH-MOX-2001, NCT03905265) with moxidectin for scabies in 2020, initially supported by funding from social impact investors and a grant from the Australian Trade Commission. This clinical trial has been delayed by the COVID-19 pandemic and remains ongoing at three sites in France and one in Darwin, Australia with results expected mid-2021 (COVID 19 dependent). A Phase 2b study is expected to begin before the end of 2021. The objective of this program is to obtain stringent regulatory authority approval for moxidectin as a treatment for scabies in adults and children, and to add this tool to the elimination of scabies. 🟡➕



# Moxidectin for lymphatic filariasis, soil-transmitted helminths, and strongyloidiasis

**Lymphatic filariasis** (also known as elephantiasis) is a painful and debilitating disease caused by filarial nematode infections transmitted through the bite of infected mosquitos. Inside the human body, the parasitic worms travel through the lymph system, often undetected, eventually causing abnormal enlargement in the arms, legs and genitalia. According to the WHO, approximately 860 million people live in endemic areas, and over 50 million people are suffering from lymphatic filariasis in tropical and sub-tropical regions throughout the world, particularly in Africa, south and east Asia and some Pacific islands.<sup>9</sup> Current community-directed treatments for lymphatic filariasis include albendazole, either alone or in combination with ivermectin or diethylcarbamazine, depending on the presence of co-endemic infections.

MDGH is collaborating in a Phase 2/3 lymphatic filariasis study being conducted in Cote D'Ivoire by the Death to Onchocerciasis and Lymphatic Filariasis (DOLF) project run by the Washington University, St. Louis, USA. This study was initiated in Q3 2020 and will evaluate the safety and efficacy of moxidectin in combination with albendazole and diethylcarbamazine.

**Soil-transmitted helminth infections** caused by roundworm (*Ascaris lumbricoides*), whipworm (*Trichuris trichiura*) and hookworm nematodes (*Ancylostoma duodenale* and *Necator americanus*), are among the most common of all infections with

an estimated 1.5 billion people infected worldwide.<sup>9</sup> Affecting the world's poorest communities, these parasitic worms are transmitted through contaminated soil where sanitation is poor. Current treatments recommended by the WHO are albendazole and mebendazole.

MDGH is collaborating in Phase 2 soil-transmitted helminth studies in Tanzania, being conducted by the Swiss Tropical and Public Health Institute, Switzerland. Proof-of-concept studies have already been completed. A study to evaluate moxidectin co-administered with albendazole in adolescents infected with soil-transmitted helminths will soon commence on Pemba Island, Tanzania.

**Strongyloidiasis** is caused by *Strongyloides stercoralis* and is another soil-transmitted helminth infection. The WHO estimates that up to 100 million people, particularly children, are infected with this parasitic worm.<sup>10</sup> The causative worm has a different lifecycle from the other soil-transmitted helminths and infection can be fatal. Treatment options are limited, with ivermectin being the medicine of choice, while other anthelmintic medicines, such as albendazole and mebendazole, lack sufficient efficacy as a single dose.

MDGH is collaborating in clinical trials in Cambodia and Laos led by the Swiss Tropical and Public Health Institute, Switzerland. 🌱+



*Woman suffering from elephantiasis (Madhya Pradesh, India)  
Photo credit: Getty Images. Used with permission.*

## Sustainable manufacturing and supply chain

The successful implementation of moxidectin as an elimination tool against river blindness requires more than demonstrating its significant superiority to ivermectin in clinical trials. It also requires the establishment of a reliable and sustainable manufacturing and supply chain for moxidectin. MDGH is currently exploring multiple options, in collaboration with major global stakeholders, to achieve that goal.

The company is sharing its experience with key stakeholders in the field of neglected tropical diseases in order to assist others in following MDGH's work. 🌱+

# CC-11050 (AMG 634)

MDGH has assumed full responsibility for the development of CC-11050 (AMG 634) from Amgen, including Phase 2b clinical trials for both tuberculosis and erythema nodosum leprosum. The compound is a PDE4 inhibitor that has shown promising results in Phase 2a clinical trials for these two diseases.

## Development for tuberculosis

Tuberculosis is caused by the bacteria *Mycobacterium tuberculosis*. The infection primarily affects the lungs, but can disseminate to any part of the body, such as the kidney, spine and brain. Despite being both a preventable and curable disease, it is the leading cause of infectious death worldwide, with 10 million new cases and 1.4 million deaths reported in 2019.<sup>8</sup> While tuberculosis is global, most people who contract tuberculosis are in LMICs.

Current treatment regimens for tuberculosis are complex, requiring patients to undergo daily multidrug treatment for up to two years. While effective in many, the complexity of the treatments as well as social factors (such as accessibility) can lead to poor compliance, drug resistance and treatment failure. Multidrug-resistant tuberculosis remains a public health emergency: of the 10 million new cases in 2019, over 200,000 were multidrug-resistant.<sup>8</sup>

The role of the immune system is critical in tuberculosis pathogenesis as people with weakened immune systems are at much higher risk of developing tuberculosis disease. While some antibacterial strategies are effective against tuberculosis, there are few immunomodulatory treatments.

CC-11050 (AMG 634) has shown promising preliminary results, now published in *The Lancet Respiratory Medicine*,<sup>11</sup> and a Phase 2b trial in tuberculosis patients in combination with the standard anti-tuberculosis regimen is about to start. This multi-country study is sponsored by the Aurum Institute, South Africa. .-++



A doctor examining the condition of a patient's lungs (Tbilisi, Georgia)  
Photo credit: Dato Koridze /STUDIO at Getty Images for TB Alliance. Used with permission.



*Patients with ENL are often separated from their families in a non-private hospital ward for long periods of time, unable to work to support their families. They may be in and out of hospital for many years.*  
**Photo credit:** The Leprosy Mission Nepal. Used with permission.

## Development for leprosy

Leprosy, also known as Hansen's Disease, remains a common, devastating condition. According to the WHO, 208,619 new cases of leprosy were registered in 2018 across 127 countries, with the largest number of cases in India, followed by Indonesia and Brazil.<sup>12,13</sup> Caused by slow-growing *Mycobacterium leprae* bacteria, leprosy may take up to 20 years before signs of infection affect nerves, skin, eyes or the lining of the nose.

Around 30 to 50% of leprosy patients also develop erythema nodosum leprosum.<sup>14</sup> This condition is an immune-mediated and severe complication of leprosy, which can affect people with active leprosy infection, as well as those that have been effectively cured with multidrug therapy, even many years later. Erythema nodosum leprosum is associated with clinical features such as skin lesions, nerve pain, joint pain, and inflammation of the eye, bone, lymph nodes and kidneys. It is treated mainly with corticosteroids, which are often required for extended periods of time, leading to serious adverse effects. While the underlying immunologic mechanisms of erythema nodosum leprosum may not be fully understood, the inflammatory responses can cause morbidity and mortality if not treated in a timely manner.

Early results from a Phase 2a study in Nepal suggest that CC-11050 (AMG 634) may resolve the symptoms of erythema nodosum leprosum and the compound is about to be evaluated in a Phase 2b clinical trial. The study, funded by Amgen, involves approximately 40 patients treated over a longer duration. 🟢➕

# Past years in review

*Highlights of the achievements since the US Food and Drug Administration approval of moxidectin in June 2018 are:*

## 2018

**JUNE** MDGH receives approval for moxidectin for the treatment of river blindness (onchocerciasis) in adults and children 12 years of age and older, as well as a Priority Review Voucher (PRV).

**OCTOBER** MDGH Founder and Managing Director, Mark Sullivan, named 2019 Victorian Australian of the Year.

**NOVEMBER** MDGH awarded the 2018 AusBiotech and J&J Innovation Industry Excellence Award.

## 2019

**FEBRUARY** MDGH named as one of Fast Company's 'World's Most Innovative Companies' for 2019.

**MAY** MDGH sells its PRV to Novo Nordisk, with funds raised going towards additional moxidectin clinical trials and field implementation.

Mark Sullivan recognised as one of Fast Company's 'Most Creative People' of 2019.

**AUGUST** A project partially funded by The European and Developing Countries Clinical Trial Partnership (EDCTP) award of €4.6m grant to a consortium coordinated by the Luxembourg Institute of Health and including MDGH in a key role, to accelerate river blindness elimination, begins. The project includes a paediatric dose-finding study and two phase 3b trials, one comparing efficacy and safety of annual and biannual moxidectin or ivermectin treatment and one providing additional safety data on single-dose moxidectin in over 12,000 patients. It also involves mathematical modelling of moxidectin- and ivermectin-based elimination strategies to support country onchocerciasis treatment/elimination policy decisions.

**NOVEMBER** Mark Sullivan recognised as EY's 'Entrepreneur of the Year' in the category of Australian Social Entrepreneur.

**DECEMBER** Atticus Medical Pty Ltd established as the commercialisation entity to develop moxidectin for indications with large market potential – such as scabies and headlice – through a joint venture between the Global Health Investment Fund (GHIF) and MDGH.

2020

2021

**JANUARY** MDGH initiates a Phase 2a moxidectin dose-finding study in people with scabies in Australia and France.

**FEBRUARY** 2020 Mitchell Humanitarian Award presented to Mark Sullivan and John Reeder (WHO/TDR).

MDGH and GHIF sign a 'Development Commercialisation Agreement' and an 'IP Management Agreement' to further develop moxidectin via Atticus Medical Pty Ltd.

**MARCH** Results of a Phase 2 clinical trial show that moxidectin, alone and in combination with albendazole, is both effective and well-tolerated for the treatment of whipworm (*Trichuris trichiura*) in adolescents.

**JULY** Mark Sullivan recognised by the Australian Academy of Technology and Engineering with the Clunies Ross Entrepreneur of the Year award.

**OCTOBER** Moxidectin nominated in 'Best Pharmaceutical Product' category for the 2020 Prix Galien USA Awards.

**DECEMBER** MDGH licenses a new molecule, CC-11050 (AMG 634), from Amgen to advance its development as a potential treatment for tuberculosis and erythema nodosum leprosum.

**JANUARY** A project partially funded by an EDCTP award of €2.8m grant to a consortium, led by the Luxembourg Institute of Health and including MDGH, starts. The project's aim is to develop a paediatric formulation of moxidectin for neglected diseases.

**MARCH** An article in The Lancet Respiratory Medicine is published by Aurum Institute and partners and suggests that combination therapy with CC-11050 might enhance the recovery of FEV<sub>1</sub>, a key measure of lung function.



## THE STORY OF MOXIDECTIN'S IMPRINT

# What's in a “debossed code”?

### Have you ever wondered what the letters and numbers stamped on the surface of a tablet mean?

Just like the license plate on a car, there often is no meaning behind the code stamped on a tablet. It's simply a unique identifier that makes each tablet readily identifiable by healthcare providers and patients. But in many countries, it IS possible to designate your own plates, and pills, with letters or numbers that are meaningful.

We wanted to do this with moxidectin, the first new treatment for river blindness in 30 years.

When we reached the point in the manufacturing process to choose the identifier code for moxidectin tablets, we chose to imprint (deboss) the letters AKKA onto every tablet. The meaning behind these four letters is very important to us: they are the combined initials of two people who have been central to the development of moxidectin for the fight against river blindness.

The AK is for Dr Annette C. Kuesel from WHO/TDR. She has been the only constant for moxidectin during its clinical development. That we even got to this point in moxidectin's development program is almost entirely due to her tenacity and passion. Dr Kuesel is continuing to provide her expertise and experience for further trials of moxidectin needed to ensure that WHO and the countries affected by river blindness have the data they need to decide whether, where, and how to use moxidectin for the elimination of this disease.

The other initials, KA, belong to the late Dr Kwablah Awadzi (June 13, 1939 – March 16, 2011). Dr Awadzi was a monumental figure in the fight against river blindness. Millions of Africans have benefitted from programs and principles championed by him, and his legacy spreads far and wide. He founded the Onchocerciasis Chemotherapy Research Center (OCRC) in Ghana and dedicated his life to researching more effective treatments



for this disease and to training the next generation of African clinical researchers. His work included clinical and community studies of ivermectin, the drug used currently by river blindness endemic countries to treat tens of millions of people each year.



Dr Annette C. Kuesel (left) and Dr Kwablah Awadzi

Dr Awadzi was a dear friend and mentor to Dr Kuesel. He called her his twin and always sensed, even across thousands of kilometres, when she needed a taste of his wonderful brand of humour. Dr Awadzi designed and led the first study of moxidectin in people with river blindness and was instrumental in designing the pivotal Phase 3 study and helping Dr Kuesel and investigators in Ghana, Liberia, and the Democratic Republic of the Congo to conduct it.

Dr Awadzi passed away suddenly on March 16, 2011. On what would have been his 79th birthday, June 13, 2018, the US FDA approved moxidectin for the treatment of river blindness in people aged 12 and older. 🌱+

***Imprinting AKKA is the small way of honouring the legacy of these two heroes who have dedicated their lives to improving human health.***

# Funding the future

MDGH has an ambitious workplan with a goal to impact the lives of more than one billion people. The company needs financial support to achieve this goal.

MDGH is a not-for-profit company registered with the Australian Charities and Not-for-profits Commission (ACNC) and is endorsed as a Deductible Gift Recipient (DGR, type 1). MDGH has been self-funded from the outset and, as such, is independent of major philanthropic funders, governments and companies. The company has preserved the right to self-determine its projects of interest.

When taking on the sponsorship of moxidectin in 2014, we were fortunate to be able to leverage the potential value of the US FDA PRV to attract funding from the social impact fund, the Global Health Investment Fund (GHIF). After receiving a PRV, we sold it to Novo Nordisk and repaid our investors. The excess of these funds continues to support paediatric and field use studies, manufacturing requirements and scabies development.

As part of the investment from GHIF, both parties agreed to pricing, quality and supply obligations for moxidectin, and also to establish and co-fund a jointly-owned company called Atticus Medical Pty Ltd. Atticus Medical is providing MDGH with some of the financial support for further development and delivery of moxidectin in river blindness and scabies. In addition, MDGH was successful in receiving two substantial grants from the European and Developing Countries Clinical Trial Partnership in 2019 and 2021. The latter is supporting the development of a paediatric formulation of moxidectin, which, when available, will represent an important advance on the current the standard of care (ivermectin), which is not available

as a paediatric formulation or for use in children weighing less than 15 kilograms.

*In the 15 years since the founding of MDGH, the company has grown, but the vision, approach and focus remain the same.*

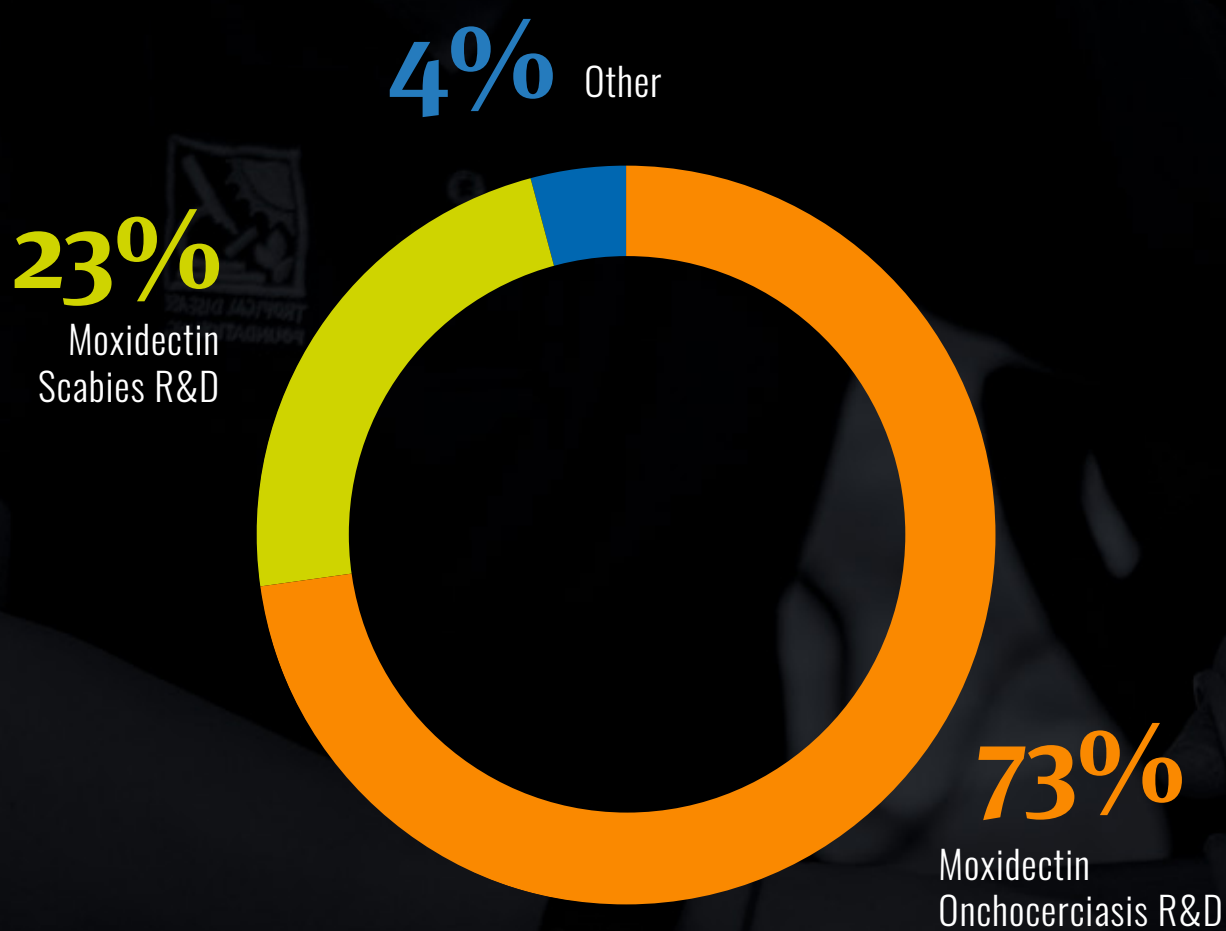
As the scope of our work and the potential for impact increases, so does the need for capital. We aim to remain innovative in the approach to fundraising and while we continue to use our financial resources and seek grants, the coming

year will see us diversifying funding sources to include philanthropic support and impact investment. We will be reaching out to supporters to raise funds for ongoing and new programs.

Detailed financial reports, including MDGH audited financial statements, are available on the ACNC website (click [here](#)). Briefly, just under three quarters of MDGH R&D funding in 2020 went to the moxidectin for river blindness program (73%), with the remainder going to scabies (~23%) and other indications (4%).

In the 15 years since the founding of MDGH, the company has grown, but the vision, approach and focus remain the same. As the development portfolio is expanded, so will our presence around the world. To that end, we will be restructuring the company's global footprint. We are in the process of establishing charity entities in the United Kingdom, the United States and the European Union. 🚀+

## Use of Research & Development Funds



# Giving thanks

We are deeply grateful to the very many people and organisations that have supported MDGH's journey so far, a list that is too vast to record. However, we would particularly like to acknowledge the support of individuals and organisations noted below given that their contribution was fundamental to MDGH's ability to achieve its objectives to date.

In 2014, MDGH licensed from the WHO the world-wide rights to moxidectin for all human health applications. The company also assumed full responsibly for moxidectin's registration for onchocerciasis as well as its development for other neglected tropical diseases. The close collaboration between WHO/TDR and MDGH has been critical in this process, with the input of Dr John Reeder and Dr Annette C. Kuesel being particularly significant.

The Global Health Investment Fund (GHIF) invested US\$13m in 2015 to support MDGH's work for completing the US FDA dossier for the registration of moxidectin for the treatment of onchocerciasis.

In 2016, MDGH was awarded AU\$1.5m grant from The Australian Trade Commission to support the clinical development of moxidectin for scabies treatment and elimination via the Australian Tropical Medicines Commercialisation Program. Matching funding was provided by GHIF.

The following foundations were also instrumental in kickstarting the scabies program with a total investment of AU\$450,000 provided to MDGH in 2017: Roberts Family Foundation; Australian Philanthropic Services Foundation Pty Ltd; Konia Pty Ltd; Este Louise Pty Ltd; Sunshine Foundation

Pty Ltd; Wolf Capital Nominees Pty Ltd; and The Crothers Walton Foundation.

In 2019 and 2021, EDCTP provided two substantial grants to MDGH and partners: €4.6m for additional clinical trials comparing efficacy and safety of single or annual and biannual moxidectin or ivermectin treatment, and €2.8m for the development of a paediatric formulation of moxidectin.

In 2020, The Bill and Melinda Gates Foundation funded our work in scoping options for the sustainable manufacture and supply of moxidectin and the broader work on a case study for changing the supply paradigm for global health medicines.

Also, in 2020, MDGH licensed CC-11050 (AMG 634) from Amgen. MDGH acknowledges the generosity of Amgen to make it possible for MDGH to take on the CC-11050 program and for their continued support to the two Phase 2 clinical trials in tuberculosis and erythema nodosum leprosum, by generously providing CC-11050 to both studies and funding the erythema nodosum leprosum study.

We are deeply grateful to you all for your investment of time and resources, and your commitment to improving public health. 🌱+

# Leadership and Governance

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## Board of Directors

**Lorna Meldrum, PhD**

Director and Chair of the Board

**Mark Sullivan**

Founder and Managing Director

**Andrew Wilks, PhD**

Director

**Richard Fiora**

Director

**Kate Antrobus**

Director

**Daren Armstrong**

Company Secretary

## Development Committee

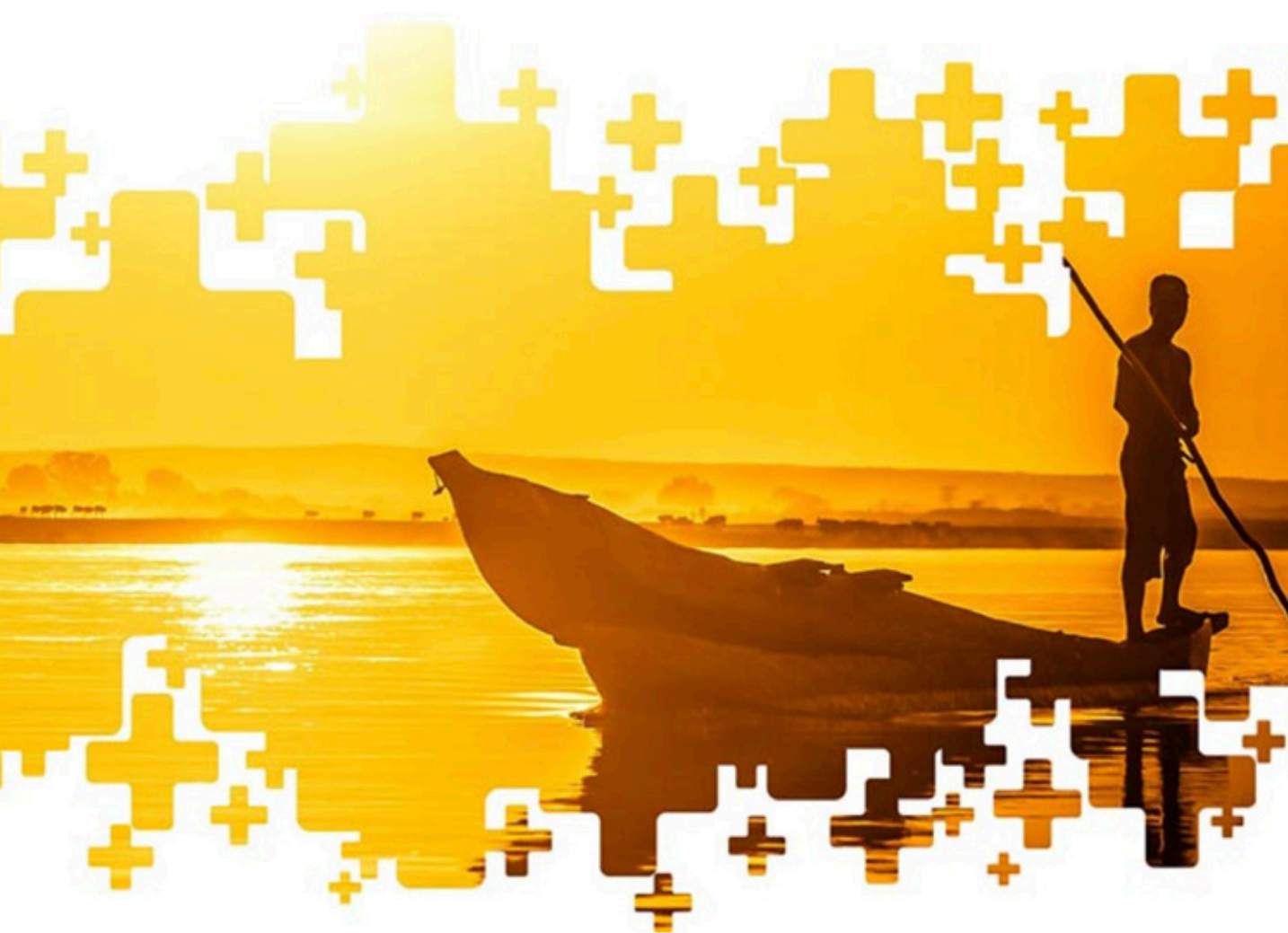
**Brian Kearney, PharmD****Fran Brown, PhD****Gerald Fisher, PhD****Joseph Camardo, MD****Ralph Smalling, MS****George Morstyn, MBBS, PhD**

*For a full list of MDGH's leadership team, please visit [medicinesdevelopment.com/team.htm](https://medicinesdevelopment.com/team.htm)*

## Endnotes

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The history, culture, diversity and value of all Aboriginal and Torres Strait Islander people are recognised, acknowledged and respected.

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