

# Care-for-Rare Foundation

Foundation for Children with Rare Diseases

From  
Discovery  
To Cure





## **Impressum**

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## Ladies and Gentlemen, Dear Friends of the Care-for-Rare Foundation,



**Christoph Klein MD PhD**  
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Chair, Department of  
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Maximilians Universität Munich,  
Founder  
Care-for-Rare Foundation

Children with rare diseases are the “orphans of medicine” – they have no voice in our world that is getting louder and louder; we do not understand their illnesses and, despite great advances in the field of medicine, most rare diseases have no cure.

The Care-for-Rare Foundation has been established in order to help children with rare diseases. Care-for-Rare is Germany's first foundation solely devoted to this cause. Brought into being by professionals from medicine and science, it pursues its mission “From Discovery to Cure” through targeted sponsorship of clinical care, research and education.

No child should be destined to die of a rare disease regardless of his or her national and ethnic background or their families financial situation!

Since its foundation in 2009, Care-for-Rare has already achieved many milestones, but we still have a long and challenging journey ahead of us. We designed this brochure to share the vision and principles of our work with you – it presents you with stories of individual children, allows you to engage yourself and offers you specific opportunities to get involved in our mission. Many people have expressed that the Care-for-Rare Foundation is unique: Care-for-Rare emphasizes the dignity of each individual child and brings people together across all borders.

I much hope that reading these pages will move you emotionally and convince you intellectually; For the Care-for-Rare Foundation can only build a future in the interest of our children with rare and common diseases if it acts in sustainable collaboration with other people and institutions. Forging strong alliances will help us to achieve our mission our mission beyond the frontiers of current medical practice and the boundaries of nations!

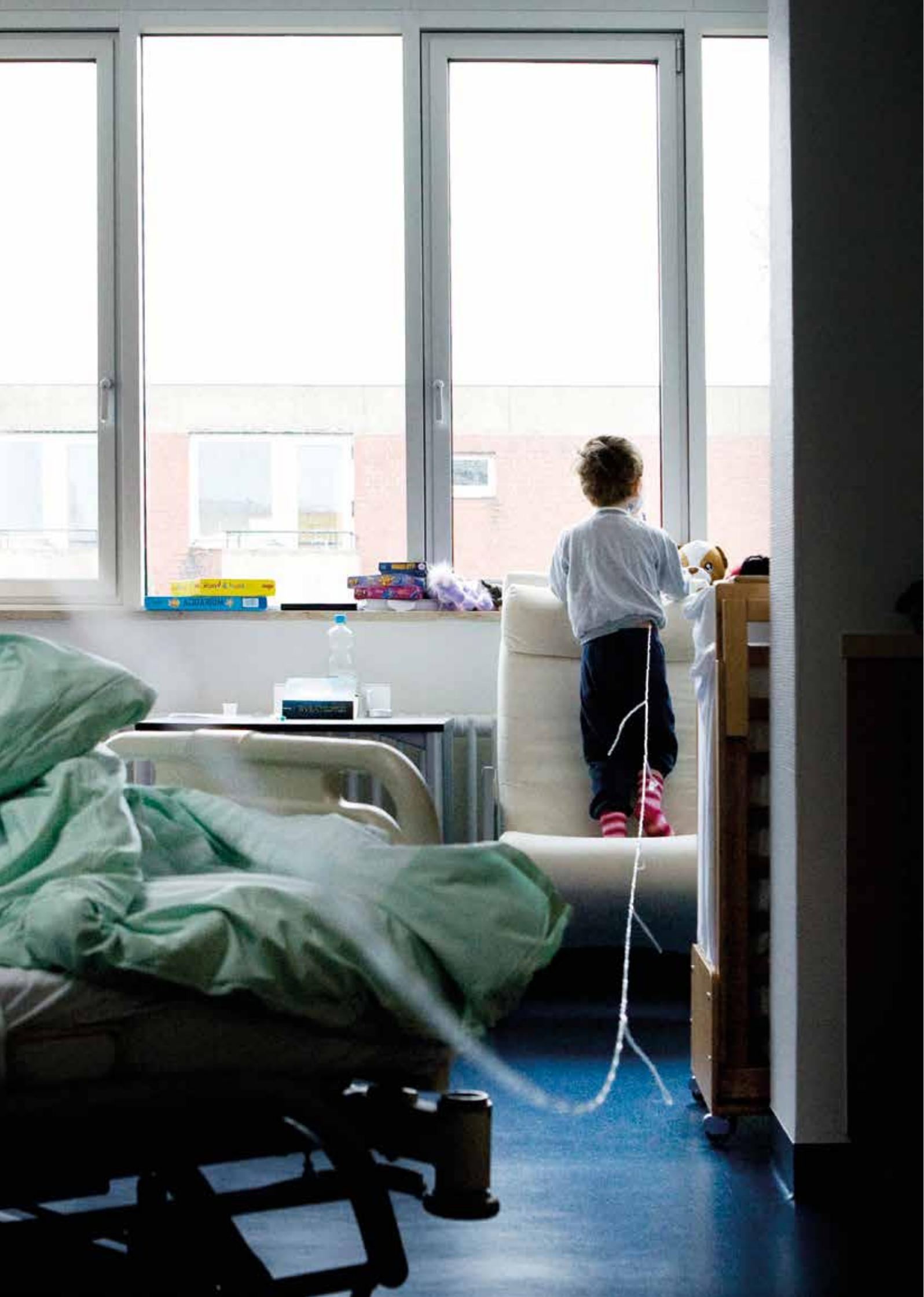


Prof. Dr. Dr. Christoph Klein



JOSEPHINE REPRESENTS  
MANY CHILDREN WITH  
RARE DISEASES.  
THEY ALL ARE HOPING FOR  
A CHANCE TO LIVE.





## Children with rare diseases – the orphans of medicine

**C**hildren with rare diseases are the orphans of medicine – their fate barely enters public consciousness. Their life of suffering is often characterized by lengthy odysseys from one doctor to another, misdiagnoses and often inappropriate or erroneous therapies. Even if the correct diagnosis is made, the patients are often confronted with the fact that there are no effective options for treatment; their diseases are too rare for the pharmaceutical industry to be interested in investing in the research and development of new drugs.

In Germany alone, over 2,000 children a year still die from the complications of their rare diseases.

Around 7,000 rare diseases are known today and this number is growing constantly. Most rare diseases develop from a small defect in the genome. Only one single element of the approx. 3.2 billion components of the human genome needs to be defective for it to result in a rare disease. The array of symptoms is very diverse; rare diseases can affect each organ system individually or in combination with each other. Some rare diseases carry the name of the doctors who first discovered them, such as the Hurler, the Kostmann, and the Wiskott–Aldrich syndrome. Others are defined by their symptoms and clinical signs, such as cystic fibrosis or ceroid lipofuscinosis, or are identified by the name of the affected gene, such as G6PC3-deficiency or IL21-receptor deficiency.

The degree of severity of the individual diseases is extremely variable. There are rare diseases that only slightly compromise the health of the person affected, and other rare diseases that are terminal within a short period of time. Until just a few decades ago, many patients with rare diseases died at a young age; nowadays, thanks to advances in medicine, they are able to survive the worst stages of their illnesses, even if they cannot really be cured completely.

But there is hope. The history of pediatric oncology has showed us that cancer can be cured. Whereas 60 years ago, all children with malignant tumours would die, nowadays four out of five children affected by cancer can be cured permanently. This milestone in medical history

shows us the way forward: we need to put this success into practice for those children whose illnesses are still incurable.

In contrast to chronic illnesses common in adulthood, rare children's diseases allow us to define what caused them in precise terms. The causes of common modern illnesses are heterogeneous and extremely complex, whereas rare diseases are usually caused by a single defective gene. Nowadays modern genome sequencing allows us to track down the underlying gene defects. This not only allows us to explain the mechanisms of the development of the diseases, but also opens up new perspectives for developing targeted ways of treating them.

The “orphans of medicine” can thus become the pioneers of a new era of genomic medicine, in which pharmaceutical ingredients, cell and gene therapy processes are deployed purposefully to cure illnesses – not just rare diseases, but also a wide range of much more common illnesses.

Research into rare diseases is not only extremely important for patients affected by these rare conditions. Scientific knowledge on rare diseases also helps illuminate the fundamental principles governing differentiation and function of cells and organs; this has implications for the basic sciences and for medicine as a whole. Research into each rare disease can at the same time open a new door towards a better understanding of disease-related processes in humans. In this way, the current orphans of medicine, children with rare diseases, can become the pioneers for the future of modern medicine.



## The Care-for-Rare Foundation

**F**rom Discovery to Cure – this guiding principle runs through the funding activities of this young and ambitious foundation seeking to make a real difference for both, the children with rare diseases, and medicine in general.

Care-for-Rare follows the vision that no child should be destined to die just because his or her disease is too rare to elicit interest of mainstream researchers or the pharmaceutical industry.

We must respect the dignity of each child and do our best to make sure that all children have access to state-of-the-art medical therapies, regardless of their ethnic and national origin and the financial resources of their families. Furthermore, we need to expand our knowledge by scientific discoveries in an attempt to nourish hope for all those children who cannot yet be cured today.

### **Vision:**

To cure all children with rare diseases – worldwide.

### **Mission:**

To establish a global alliance in order to identify the genetic causes of rare diseases and to develop effective treatments, following a three-stage approach: recognize – understand – cure.

### **Values:**

The Care-for-Rare Foundation builds bridges between doctors, researchers, patients, political representatives, business, the media and art. Every child should have access to medical care, regardless of their ethnic, religious or financial background. The Care-for-Rare Foundation complies with both national and international guidelines for the work of charitable organisations.

### **Strategy:**

The Foundation currently has five specific areas of funding. The international Care-for-Rare Foundation unites physicians and scientists from many institutions and countries in our joint mission to investigate the causes of rare diseases and develop new treatments for them. The Care-for-Rare Academy supports further education through short- and long-term training courses. In individual cases Care-for-Rare Aid helps young patients facing acute emergencies. Care-for-Rare Awareness supports campaigns that focus public attention on the lot of children with rare diseases and sensitize medical staff to the issue. This should lead to a more rapid and precise improvement in the area of diagnoses and treatments. The Care-for-Rare Awards honors outstanding scientists who have already achieved success in the research of rare diseases.

Care-for-Rare Foundation  
**Alliance**



The international **Care-for-Rare Alliance** unites physicians and scientists in many institutions and countries in their joint mission: to understand the causes of rare diseases and to develop novel therapeutic strategies. Through their joint research activities they share their vision that children with rare diseases should no longer be overlooked as “orphans of medicine” but rather become the avant-garde of a new era of genomic medicine.

The flagship of these activities is centered on the Dr. von Hauner Children’s Hospital at the Ludwig-Maximilian-Universität Munich. Founded in 1846, Bavaria’s oldest and most renowned pediatric hospital has guaranteed excellence in clinical practice, research and teaching for many generations. Caring for children with rare diseases has become a prime focus, over a third of all patients followed are suffering from one of the 7,000 rare diseases. Our Care-for-Rare Centers provides state-of-the-art care and is able to carry out cutting-edge research in conjunction with many partners worldwide.

The Dr. von Hauner Children’s Hospital homes the Head Office of the government-funded National Networks of Rare Diseases and orchestrates multiple national and international scientific consortia.

The Alliance associates centers of excellence in Europe and the United States, such as Boston Children’s Hospital, SickKids Toronto, Institut Pasteur Paris and the Institute of Molecular Biotechnology Vienna with academic pediatric centers in emerging countries. Together, we can make a difference for children with rare diseases across all borders!



# Care-for-Rare – Beyond All Boundaries



**A** growing number of academic centers worldwide engage in the Care-for-Rare mission. Whereas the network's current emphasis is still placed on rare diseases of the blood and immune system, other catastrophic pediatric diseases will come into focus in the future. A current partnership with the German Academic Exchange Service allows us to intensify scientific exchange with Care-for-Rare partners in Boston (Children's Hospital, Harvard Medical School), Toronto (SickKids), Vienna (IMBA), Kayseri (Erciyes University), Tel Aviv (Sheiba Medical Center), Isfahan (University of Medical Sciences), and Bangkok (Chulalongkorn University). Thanks to our strategic collaboration with the Helmsley Charitable Trust in New York City, Care-for-Rare is able to maintain a global network for research into rare intestinal inflammatory disorders in children. The Alliance also provides a valuable platform for educational activities, in particular in less privileged countries such as Morocco or Columbia.











Felix is one of the first patients worldwide who underwent stem cell gene therapy

## Felix and the development of gene therapy

**F**elix comes from Rhineland-Palatinate; he is now 10 years old. When he was just a baby, he had to be “wrapped in wool” because any slight injury would lead to serious bleeding due to a low platelets count. Felix’s pediatrician initially diagnosed him with “idiopathic thrombocytopenic purpura” – a common disease in children which could be cured without serious complications in most cases. Felix thus shared the lot of a large number of children with rare diseases, who are initially treated for a misdiagnosed condition. At university, trainee doctors still learn that rare diseases are so rare that they will never come across them in their day-to-day practice. However, Felix’s pediatrician was sceptical – he referred him to a centre where the correct diagnosis of Wiskott–Aldrich syndrome was finally established. Wiskott–Aldrich syndrome is a very rare disease which only affects one in a million children. Due to a small mutation on the X chromosome, the children affected demonstrate a tendency to bleed heavily, tend to catch life-threatening infections and also suffer from serious autoimmune diseases. The only effective treatment for Felix was to transplant healthy blood stem cells, but a suitable donor could not be found. Felix’s parents therefore decided to let him participate in a clinical trial, which at that point in time was the first in the world to test the hypothesis that DNA repair of Felix’s own stem cells could cure the disease. Felix was one of the first patients to undergo this experimental stem cell treatment and it was remarkably successful. Shortly after the re-infusion of his own blood stem cells which had been treated with gene shuttles, his body was producing enough platelets and the tendency to bleed excessively disappeared. Felix’s immune system had also been strengthened and was robust enough to fight off potential pathogens and to balance the imbalance

typical of autoimmune processes. Felix was able to lead a healthy life for many years without needing to be wrapped in cotton wool.

Admittedly, after a few years Felix showed serious side effects in the form of myelodysplastic syndrome, a precursor of leukaemia; this was similar to other patients following stem cell therapy with the first generation of gene shuttles. Fortunately, a new search for a suitable bone marrow donor on the international data bank was successful this time round. Felix finally underwent a transplant, which then also cured the side effects associated with the gene therapy.

Felix’s story illustrates very clearly that the paths to curing patients with rare diseases are not always straightforward. Essential and urgent new treatments are being developed little by little and on this journey, setbacks are unavoidable. Nevertheless, Felix played a decisive role in a development which is still by no means finished. A large number of scientists are working under great pressure to develop new gene shuttles which cause fewer side effects, as well as on new genetic micro-surgery technologies, which could potentially make the use of conventional gene shuttles redundant.

Felix is doing well nowadays - thanks to his amazing family who gave him the strength and confidence he needed - his disease has been overcome and his life lies in front of him with all its many opportunities.

## Sevkan and the development of new therapies for severe inflammatory bowel diseases

**S**evkan is a Turkish boy; he has struggled with serious bowel disease since he was one year old. Unbearable stomach aches and purulent abscesses alongside a high temperature were the reasons he had spent three of his six years of life in hospital. The family consulted more than a dozen different doctors and hospitals; the young boy, whose parents are originally from Turkey, was forced to undergo countless examinations, but no one could find an explanation for this extraordinarily severe illness. However, the lack of diagnosis that weighed on the family, was made worse by the fact that none of the various treatments showed any sign of success. Sevkan had to have numerous surgeries; one day he almost lost his entire colon – but the inflammation smouldered on and on.

When the team at the Care-for-Rare Foundation was consulted and it became clear that the knowledge contained in the medical textbooks had been exhausted, new ground was broken. Under the presumption that the cause of Sevkan's illness could be found in a small genetic mutation, a research project lasting several years was initiated.

It took three years to solve the mystery: the severe bowel inflammation did not result from an erroneous function of the intestinal cells but rather from the inability of Sevkan's immune cells to recognize the harmless bacteria in the bowel as harmless. The consequences were horrendous: the immune system thought that all the

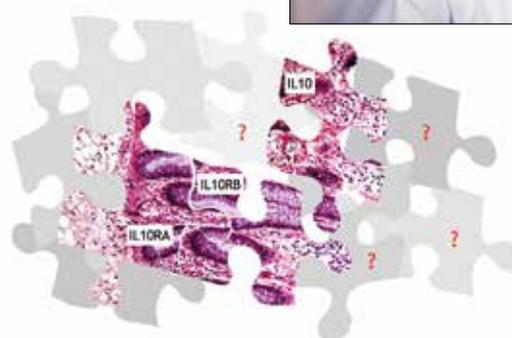
bacteria in the bowel should be combatted and destroyed the whole organ due to the uncontrolled inflammatory reaction. This discovery saved Sevkan's life, since a new promising approach to treatment suddenly seemed feasible. If it was possible to replace the entire immune system by transplanting appropriate blood stem cells, then the defective antenna function would be replaced. Sevkan's doctors, in conjunction with Sevkan and his family, decided to take this experimental course of treatment. Its success was awe-inspiring: within one month after the treatment, the infection healed and Sevkan was finally able to lead a normal life with his family and friends. Today, he is a healthy teenager and is on the way towards becoming a mechanic in car manufacturing.

But this did not just affect Sevkan; dozens of other children all over the world were able to benefit from these findings. Sarah from Munich, Ben from Boston, Thuraja from Bangkok and many, many others were suffering from this new disease due to a defect in the interleukin-10 receptor. It was possible to cure them all by means of blood stem cell transplants.

Furthermore, the stories of Sevkan, Sarah, Ben and many others have revealed that there are far more children in the world with similar severe inflammatory bowel diseases whose causes remain unexplained. But thanks to a newly established global network, the Care-for-Rare Foundation is now making a contribution to understanding their diseases as well. This research is driven by the hope that one day, all the other children affected can also be cured.

## Impact Assessment

The results of the research funded under the auspices of the Care-for-Rare Alliance are recognized and appreciated by the scientific community. Although there are no generally accepted criteria for impact assessment in the area of research into rare diseases, the scientific articles that have emerged with the help of the Care-for-Rare Foundation have been widely disseminated in prominent professional journals for the most part (New England Journal of Medicine, Nature Medicine, Nature Genetics, Science Translational Medicine, Journal of Experimental Medicine, Gastroenterology and Blood, among others). As a result of these publications, a large number of doctors and scientists around the world have been able to share in the results and thus provide their respective patients with a precise diagnosis and also offer new treatment options. Furthermore, a large number of crystallisation centres for new scientific initiatives and projects have come into being, and the results of these activities will certainly have an impact on our understanding of rare, as well as common diseases.



Sevkan and Äyâ stand for many children with early onset inflammatory bowel diseases whose genetic defects are only beginning to be understood. Solving these mysteries is like a puzzle – the more pieces emerge the better the understanding of the complex pathways controlling tolerance and immunity in the gut.



Care-for-Rare Foundation  
*Academy*

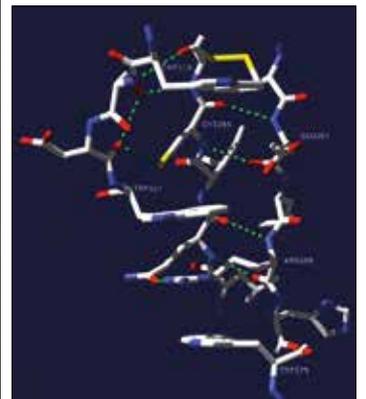


**T**he **Care-for-Rare Academy** aims to help young doctors and scientists acquire skills in the interdisciplinary care of sick children and to learn how to shape the future of medicine.

Under the motto “Allow time for thinking” the Foundation awards study grants and funds further education of physician-scientists in pediatric sub-disciplines. Since the costs of this highly-specialised training are not currently covered by either the health insurance companies or the hospital authorities in Germany, the Care-for-Rare Foundation helps ensure that excellent clinical care is available to sick children. In addition, Care-for-Rare supports gifted young physicians and scientists from less privileged countries for in-depth education and training in centers of excellence.

As a result of the funding from the Care-for-Rare Academy, a new generation of doctors and scientists are learning how novel principles of genomic medicine can be developed and employed to benefit sick children. In this way, the specific individual characteristics of the patients can be taken into account and a tailor-made approach to medical care made possible. The Foundation invests in the brightest minds and aims to help the young scientists discover and hone their talents.

Alongside developing concepts for further discipline-specific education, we are also committed to promoting critical reflection on a philosophical meta-level. The clinical practice of medicine and the focus of science need to be constantly re-examined in the light of ethical principles. Since specialist disciplines of natural sciences often neglect this ethical dimension, the Care-for-Rare Foundation aims to make an important contribution in this respect, so that doctors and scientists all over the world are always committed to carrying out medicine with a human touch – and deep respect for human dignity.



Selim and his sister are the first patients with inherited GCSFR deficiency, a new disease discovered by Care-for-Rare fellow Alexa Triot.

## The discovery of the G-CSFR defect

**A**lexa has recently graduated from Harvard Medical School in Boston. Even as a medical student, she already wanted to expand her horizons through a year of academic study abroad. The young American registered at LMU and spent a year at the Dr. von Hauner Children's Hospital – supported by the Care-for-Rare Foundation. As a passionate researcher, she successfully identified the cause of disease in two Turkish children who repeatedly developed bacterial infections as a result of a lack of the white blood cells, neutrophil granulocytes. Selim and his sister Leyla live in Turkey, but their disease is being studied in Germany as a project of the international Care-for-Rare Alliance.

Under the supervision of experienced scientists in the Care-for-Rare Laboratories, Alexa not only learned the general principles of scientific work, but also the specific molecular and cell-biological methods to explain rare diseases in children. Alexa identified the first patient in the world with inherited mutations in the gene of the G-CSF receptor: the mutations lead to incorrect folding and thus to insufficient expression of the receptor on the surface of the cells. This discovery means that we can now look for new pharmacological agents which will partially or fully correct the malfunction of the G-CSF receptor. These new active substances can potentially be used to treat other diseases resulting from defective protein folding.

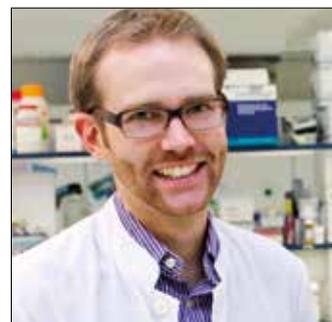
The Care-for-Rare Foundation funds “clinician-scientists” in particular, that is, doctors who, as a result of their distinguished scientific education, are capable of thinking beyond the frontiers of knowledge and to constantly stretch the boundaries of medicine in the interest of children with rare diseases.

*” Working with C4R was a great opportunity to realize the responsibility and capacity that we have as physician-scientists to better understand and develop new treatment strategies for children with under-represented diseases. The interface between the patient and lab provided an efficient way to translate theoretical concepts into understanding the pathophysiology of their disease, as well as develop real-life treatment strategies.“*



## Care-for-Rare Long-term Fellows

Whereas there are many funding opportunities for young scientists, the options for specialized training for physician-scientists are very scarce. Therefore, Care-for-Rare specifically focuses on this group and provides funds to secure innovation and creativity – critical prerequisites needed to change the future of children with rare diseases.



**Dr. Tilmann Schober, Dr. von Hauner Children's Hospital 2013-2016**  
Tilmann Schober trained in pediatrics at Hannover Medical School and the Johann Wolfgang von Goethe University in Frankfurt. Driven by passionate curiosity, Tilmann wants to understand the basics of human immunity. Funded by the Care-for-Rare Foundation, he currently investigates a recently discovered new genetic defect. He is also involved in shedding light on the mysteries of pediatric stroke disorders.

**Dr. Daniel Kotlarz, Dr. von Hauner Children's Hospital 2012-2016**  
Daniel Kotlarz is a clinician-scientist working on primary immunodeficiency diseases. He is currently supported by the Care-for-Rare Foundation to establish a global interdisciplinary network of clinicians and scientists to unravel the mechanisms of inflammatory bowel diseases in children. His outstanding work has been awarded by several national and international

awards, such as the John Harries Prize and the Science Prize of the German University Hospital Association.

**Ehsan Bahrami MSc, Dr. von Hauner Children's Hospital 2012-2016**  
Ehsan Bahrami is a graduate from Isfahan University of Medical Sciences in Iran. He was awarded a Care-for-Rare Fellowship to investigate the molecular pathomechanisms of a newly discovered gene defect associated with progressive bone marrow failure.

**Dr. Judith Feucht, Memorial Sloan Kettering Cancer Center 2015-2016**  
Judith Feucht from Tübingen University is interested in immunotherapies to fight rare pediatric cancers. She was recently awarded a Care-for-Rare Fellowship for further postdoctoral training in Michele Sadelain's lab at the Memorial Sloan Kettering Cancer Center in New York, one of the global pioneers of gene therapy of the immune system.

**Dr. Tobias Schwerd, Oxford University 2013-2014**  
Tobias Schwerd is a graduate from LMU Munich and started his pediatric residency training in 2010. He follows the Hauner Duo career track. A Care-for-Rare Fellowship enables him to study principles controlling immunological tolerance at Oxford University, UK.



**Dr. Volker Wiebking,  
Stanford University 2016-**

Volker Wiebking trained in pediatrics at the Dr. von Hauner Children's Hospital. He wants to develop new gene therapy strategies for rare pediatric disorders. Volker was recently awarded a Care-for-Rare Fellowship to join Matthew Porteus's lab at Stanford University where he will design new technologies for stem cell engineering.

**Dr. Anna-Lisa Lanz,  
Oxford University 2014-2016**

Anna-Lisa Lanz finished her pediatric residency at the Dr. von Hauner Children's Hospital. Funded by the Care-For-Rare Foundation she joined Oreste Acuto's lab at the Sir William Dunn School of Pathology in Oxford UK to work on the development of T cells.



**Dhaarini Murugan MSc,  
MHH and LMU 2010-2014**

Dhaarini Murugan graduated from Madurai University, India, and was awarded a PhD at Hannover Medical School. She helped to discover new human genetic defects and contributed to our understanding of the IL-10 pathway in colitis. Dhaarini authored several scientific manuscripts published in major peer-reviewed journals such as the New England Journal of Medicine, Blood, Gastroenterology, and the Journal of Clinical Immunology. Currently, she is a postdoctoral fellow at the Knight Cancer Institute, Oregon Health State University, USA.

**Dr. Vera Binder, Harvard Medical  
School 2014-2016**

Vera Binder trained at the Dr. von Hauner Children's Hospital, the Heinrich-Heine University Düsseldorf, the Weizman Institute in Israel and at Harvard Medical School, where she was funded by the Care-For-Rare Foundation to work on the biology of the hematopoietic stem cell niche and its role in nurturing leukemia cells. She recently published her insights in the journal Nature and returned to Munich to set up her laboratory with continuous Care-For-Rare support.



**Dr. Maximilian Witzel, Dr. von Hauner Children's Hospital 2013-2016**

Maximilian Witzel initiated his clinical pediatric training at the University of Heidelberg. Max joined the LMU to combine clinical care with scientific work on gene therapy. His published the comprehensive results of the world's first gene therapy study for Wiskott-Aldrich syndrome in Science Translational Medicine and currently investigates rare disorders of neutrophil granulocytes.

# Care-for-Rare Foundation Aid



**T**he **Care-for-Rare Aid** Program aims to give needy children with rare diseases a chance of recovery by means of tangible help in individual cases. We repeatedly receive enquiries from doctors and parents all over the world; we are not always able to make the impossible happen. However, children with rare diseases can often benefit directly from international and interdisciplinary collaboration. For this reason, the Care-for-Rare Foundation regularly collects donations in order to grant children life-saving access to modern medicine – in situations not supported by health insurance organizations. This gives them the opportunity of accessing modern genetic diagnostics, which is not reimbursed by the health insurance companies, or participating in new therapeutic procedures, the costs of which are not yet covered. For children in various parts of the world, this help can mean life-saving care not available in their home countries.



Little Knowah from the Philippines with his doctor and parents during his treatment in Munich

## Knowah's cure

**O**ne-year-old Knowah from the Philippines plays vivaciously and is happy if someone comes into his room in the Dr. von Hauner Children's Hospital. He plays with small toy cars and takes great pleasure in throwing them out of his cot over and over again. But the lively impression that this little boy gives does not hide the fact that Knowah is seriously ill. Terminally ill. He has Wiskott–Aldrich syndrome, a very rare immunodeficiency, which causes acute eczema and life-threatening infections. In addition, he experiences severe bleeding primarily in the bowel as a result of having a dangerously low platelets count. The only therapy that can save Knowah is a blood stem cell transplant. No suitable donor has been found; therefore, his parents will act as donors. They are from the Philippines and have a real odyssey behind them: "At just three days old, Knowah had a high temperature," his mother Dindin explains. The child did not sleep, he repeatedly vomited the milk that he was given, he had eczema in the oral cavity. And he constantly had a high temperature. The doctors started with the assumption that he had a milk allergy; then they thought it was amoebic dysentery; a blood test showed that something was wrong with his haemoglobin. The baby then got a lung infection.

Dindin and her husband, Joubert, went from doctor to doctor and from hospital to hospital; they finally discovered that Knowah was probably suffering from Wiskott–Aldrich syndrome. And it was clear that it was not possible to treat their child in their home country.

Research on the internet showed Knowah's parents where their child could receive help. On Christmas Day 2014, they wrote an e-mail to Professor Klein, the Director of the Hauner Children's Hospital at Munich University. The Care-for-Rare Foundation initiated a fundraising campaign to cover the costs for Knowah's treatment. Munich is a good place for treating the small boy. Not only do the physicians in Christoph Klein's team have a great experience in the area of stem cell transplants, but Knowah's condition was also described

for the first time at the Dr. von Hauner Children's Hospital in 1937 by its previous director, Professor Alfred Wiskott.

As a result of the amazing wave of support from a large number of small and large-scale donors, the money needed for Knowah's treatment could be raised. In July 2015, after the necessary preparations had been made, the little boy from the Philippines received a transplant of stem cells from his father. Fortunately, he did not experience any of the dreaded possible side effects, and four months after the treatment, Knowah is a jolly little boy who uses his charm to good effect and regales the doctors and nurses with words in German. Knowah's parents are very happy: "The doctors in Munich saved Knowah's life – we are extremely grateful for the help provided by the Care-for-Rare Foundation!"

Knowah stands for many children with rare diseases that could not be cured in their local towns or home countries. Similarly, children from Germany, Iran, Israel, Morocco, India and Pakistan, Russia, Lebanon, Hungary and Turkey, Australia and the USA have already benefited under the auspices of the Care-for-Rare Aid Programme.

Care-for-Rare Foundation  
**Awareness**



**W**ith the funding line **Care-for-Rare Awareness**, the Foundation undertakes to raise wider public awareness of the concerns and needs of children with rare diseases.

We aim to help stop these veritable odysseys experienced by children with rare diseases and instead enable rapid diagnoses to be made and the best possible care to be administered by experienced physicians. In conjunction with national and international parents' initiatives, our activities target the lay public as well as the medical community. It is imperative that physicians recognize the specific challenges of children with rare diseases and consult their highly specialized colleagues. Since most people who are not directly confronted with the destiny of a child with a rare disease can hardly imagine the burden of illness, we wish to lend our voices to the orphans of medicine.

## The story of “Precious Life”



Dr. Raz Somech treats children with rare diseases at the Sheba Medical Center of Tel Aviv University. One day, he heard of a family from Gaza whose baby Mohamad was suffering from a severe immunodeficiency. The medical possibilities in the Palestinian Territories are limited; the child had no chance of surviving. Raz Somech is a friend of the journalist Shlomi Eldar, who was calling for donations to fund Mohamad’s treatment in Tel Aviv via the Israeli media. A Jewish benefactor called in and provided the required funds. Little Mohamad from the Gaza Strip can now be treated in Tel Aviv – and the story of this treatment will be recorded in a documentary film. “Precious Life” shows a great deal more than the heartbreaking tale of a child with a rare disease. The film bears witness to the humanity that bridges the deep divide that exists between Jews and Palestinians. It is about the divisions and doubts in the face of the reality of the Intifada and the response of the Israeli army. This schizophrenic situation culminates in the question: How would Mohamad’s parents behave if their son whose life was saved in Tel Aviv was called upon to carry out a suicide attack in Israel? The award-winning film which has been nominated for an Academy Award, cannot give answers or solve the conflict in the Middle East. But “Precious Life” gives us hope. It champions universal respect for the value of each human being through the example of a child with a rare disease.



“Precious Life” to be premiered at Munich’s BMW World in the presence of film directors and protagonists

The Care-for-Rare Foundation held the German premiere of “Precious Life” with the director and the protagonists in attendance. More than 500 invited guests at BMW Welt were touched by this story; after this premiere, they all know about the concerns and needs of the orphans of medicine, but also about the new opportunities that constantly arise when people find the way into each other’s hearts.

In addition, the Care-for-Rare Foundation constantly sponsors public campaigns in order to raise awareness in society regarding the concerns and needs of sick children. On the occasion of the German-Turkish Science

Year 2014/15, we created sensitive portrait photos of German and Turkish children with rare diseases. An exhibition of these photos was made available to a wide audience in Istanbul, Ankara, Kayseri and at Munich Airport. A photo-book with pictures of children with rare diseases is currently being produced; this book seeks to show that each child deserves our respect, regardless of their nationality. Our photographers are therefore travelling the continents and documenting the fortunes of the orphans of medicine with their cameras. In a range of media including print, radio and television, the Care-for-Rare Foundation regularly reports on children suffering from diseases that still have no cure. It is not easy to measure the effectiveness of these awareness campaigns, but we are convinced that with them, we are reaching people who are sensitive and willing to engage with sick children in an entirely new mindset.

However, it is not only the general population that should be alerted regarding the needs of children with rare diseases; doctors also need to know more about these diseases so that they are able to diagnose their patients quickly and with confidence and, if possible, select effective courses of treatment for them.

The Care-for-Rare Foundation was instrumental in the establishment of a new network in South-East Asia for children with rare diseases affecting the immune system. In collaboration with the International Patient Organisation for Primary Immunodeficiencies (IPOPI), the Foundation extended invitations to attend the first European Southeast Asian Conference for Congenital immunodeficiencies. Doctors and hospital representatives as well as scientists from Thailand, Vietnam, Malaysia, Indonesia, Singapore, Hong Kong and the Philippines came together at Chulalongkorn University Hospital to lay the foundation for a Southeast Asian network for primary immunodeficiencies. The speakers not only demonstrated the latest innovations in clinical care and research, but they also set these in perspective in terms of the specific regional characteristics of South-east Asia. The attendees agreed on a common strategic

goal. It was necessary to ensure, with the help of improved regional collaboration, that patients have more rapid access to correct diagnosis and that treatment options be optimized. As a result of the involvement of the Thai Ministry of Health, concrete plans were drawn up for the Kingdom of Thailand. At the end of this historic meeting, all those involved signed a call for action and declared themselves in favour of the foundation of a Southeast Asian Society for Primary Immunodeficiencies as soon as possible.

The commitment of the Care-for-Rare Foundation shows how people come together across geographical borders to create a better future for the benefit of the orphans of medicine.

At the first European Southeast Asian Conference for Congenital Immunodeficiencies



Care-for-Rare Foundation  
**Awards**



Recognition, praise and scientific distinctions can motivate people to perform at their peak. The **Care-for-Rare Award** program was created to further direct the interest of physicians and scientists towards the fortunes of children with rare diseases. As a result of collaboration with other foundations, Care-for-Rare awards two prizes every year. The Dr. Holger Müller Prize, which is worth 5,000 euros, is presented to an author of an outstanding scientific publication, while the Care-for-Rare Science Award, endowed with 50,000 euros, provides competitive seed-funding for an outstanding research project in the field of rare diseases.

The Care-for-Rare Science Award is presented together with the Bavarian Funders' Prize under the patronage of His Royal Highness, Duke Franz of Bavaria. The award ceremony is part of the initiative "pro.movere", launched by Care-for-Rare to increase the spirit of civic engagement. Both award winners receive a bronze sculpture symbolizing two halves of a tree that fit together. This allegory suggests that science and philanthropy are intricately intertwined.





Festive award ceremony at illuminated Nymphenburg Palace and presentation of Bavarian Funders' Prize to Eva-Luise and Horst Köhler (2013) as well as José Carreras (2014)



## Sulin's legacy

Sulin, a girl of Lebanese descent living in Germany, reminds us that children with rare diseases are dependent on doctors who are not content with the current state of knowledge but rather are prepared to seek new approaches. Sulin was first referred for a liver transplant. Her doctors diagnosed her with idiopathic liver fibrosis – an inexplicable (idiopathic) scarring (fibrosis) of the liver and wanted to replace the non-functional organ with a healthy donor liver. However, a number of doctors of the Care-for-Rare team were not convinced by this diagnosis and investigated further to find the source of this inexplicable disease. They felt vindicated in their doubts when a deeper analysis of the tissue samples revealed that the liver fibrosis could not be idiopathic, but had developed from a severe bile duct inflammation through cryptosporidium – small parasites which occasionally cause temporary bouts of diarrhea in healthy human beings, but which can have fatal consequences in patients who suffer from congenital or acquired immune deficiencies. Since Sulin clearly did not have any of the known types of immunodeficiency disorders, the team in the Care-for-Rare Laboratories carried out genome-wide sequencing studies and found a mutation associated with a defective antenna on Sulin's immune cells. Further tests confirmed beyond a doubt that Sulin's illness was caused by the malfunctioning of the receptor known as Interleukin 21; she was thus the first patient in the world to receive this diagnosis. Unfortunately, however, her disease was already so advanced and a large number of organs were so damaged that, despite intensive efforts, it was no longer possible to cure her. Sulin died from her illness at the age of 13. However, she left behind a legacy: as a result of the discovery made by the Care-for-Rare Team, it was now possible to diagnose Sulin's illness in a timely fashion in a large number of other children all over the world, so that the transplantation of blood stem cells was established as the lasting cure for this rare immune deficiency syndrome. For their scientific work, Dr. Daniel Kotlarz and Dr. Natalia Zietara received not

only the Dr. Holger Müller Prize, but also the Innovation Award of German Medical Research 2015, with the following justification: "In their work Dr. Daniel Kotlarz and Dr. Natalia Zietara deal with the translational use of innovative genetic diagnostic sequencing techniques in primary immunodeficiencies. The successful transfer of theoretical knowledge to the area of patient care is of fundamental importance to academic medicine.

The Care-for-Rare Science Award has been presented since 2013 as a result of a fruitful collaboration with the Werner Reichenberger Foundation. The Awardee is chosen in a competitive selection procedure by the Foundation's scientific advisory board. Scientists from the Universities in Cologne, Munich, and Ulm have so far been enabled to initiate innovative research projects targeting rare childhood diseases.



Mutated IL21 receptor is no longer expressed at the cell surface - for their discovery of this previously unknown disease, Daniel Kotlarz and Natalia Zietara received the Holger Müller Prize 2013.

## What people say about us

### **Annette Schavan, Ambassador of the Federal Republic of Germany**



I am delighted to have taken over the patronage of the Care-for-Rare Foundation. Rare diseases are a great challenge for medicine, science and society. Care-for-Rare is a ray of hope - it operates across national borders. Humanity, scientific excellence, an interdisciplinary approach are the guarantors for its sustainable work around the world. I hope that a great number of committed people will accompany and support us on this important mission.

### **Cardinal Reinhard Marx, Archbishop of Munich and Freising and Chairman of the German Episcopal Conference and of the Commission of the Bishops' Conferences of the European Community (ComECE)**



Jesus put children first and foremost. Children with rare diseases are amongst the weakest of the weak in our world. The international Care-for-Rare Foundation stands up for them – we should support their mission to the best of our ability.

### **Claus Hipp, Entrepreneur**



Children are our future; those people who commit their lives to helping sick children are the heroes of our era; they need our support.

### **Pep Guardiola, soccer coach**



We need people with vision and energy. The Care-for-Rare Foundation at the Hauner Children's Hospital in Munich has people like this and I am delighted that they apply their vision and energy for the benefit of sick children all over the world!

### **Dr. Norbert Lammert, President of the Federal Parliament, Germany**



Children with rare diseases need the help of our government-run social security systems, but they also need increased support in the form of compelling civic involvement. The representatives of our state institutions are aware of their duty of care, but state institutions have only limited potential. Private individuals, companies and institutions committed to the common good can and should increasingly campaign where the public authorities have reached their limits. The Care-for-Rare Foundation is guided by the vision that no child should die from a rare disease and does a great job in the interest of the weakest of the weak!

### **Professor Dr. Heinrich Bedford-Strom, Regional Bishop of the Evangelical Lutheran Church in Bavaria and President of the Council of the Evangelical Church in Germany**



The Care-for-Rare Center at the Dr. von Hauner Children's Hospital is an important place for our society. The virtue of charity is seen materialize here; doctors, scientists and nurses show their commitment to sick children here and underline through their work that the dignity of all human beings is a priority in the field of medicine.

### **Prof. Dr. Horst Köhler, Former President, Federal Republic of Germany**



Children with rare diseases, the orphans of medicine, cannot make themselves heard alone in today's noisy world. They need our help and support. The Care-for-Rare Foundation is a remarkable Foundation which takes its humanitarian mandate in close proximity to medical science very seriously and thus makes an important contribution across international borders towards allowing children with rare diseases to harbour the hope of being cured.

**Joachim Gauck,**  
**President of the Federal Republic of Germany**



What particularly moved me is that such a renowned scientist humbly pointed out the boundaries of his power and that he is aware of this human dimension. I think that is magnificent. *In August 2010 on National Television Broadcasting NDR ("The best in the North")*

**Dr. Joachim Faber**  
**Chairman, Board of Directors of the German Stock Exchange Group**



Care-for-Rare is a unique Foundation with a timely mission and convincing strategy. Driven by humanitarian values, its focus on children with rare diseases leverages relevant insights into disease mechanisms which ultimately will provide cures for patients all around the world.

**Dr. Ludwig Spaenle,**  
**Bavarian Minister for Education and Culture, Science and the Arts, Munich**



The Care-for-Rare Foundation at the Dr. von Hauner Children's Hospital makes a fantastic contribution to the research into rare diseases in children and to international networking. It provides important support for the field of science in Bavaria.

**Dr. h.c. Charlotte Knobloch, Chairwoman of the Jewish Community and former Vice-president of the World Jewish Council**



The Care-for-Rare Foundation at the Dr. von Hauner Children's Hospital demonstrates time and again that medicine and the community as a whole should not be satisfied with the status quo. In the interest of all sick children, we must instead ensure that we can open up a bright future for our children, regardless of religion or national origin and with respect for the dignity of each and every person.

**Barbara Stamm,**  
**President, Bavarian Parliament**



The Care-for-Rare Foundation at the Dr. von Hauner Children's Hospital in the Bavarian capital city of Munich campaigns for the comprehensive medical, nursing and psychosocial care of children with rare diseases who live in Munich and Bavaria and from much further afield. The Foundation constantly develops creative ideas in order to enable new avenues of help for sick children to be opened up, at a time when it is an unfortunate fact that we cannot always take it for granted that the sick child is placed at the centre of attention. Without the solid backing of reinforced civic involvement this key pillar of support would not be sustainable.

**His Serene Highness**  
**Prince Karl Friedrich of Hohenzollern**



I am familiar with the fate of children with rare diseases as a result of personal experience in our own family. We want to ensure that no more children need to die from rare diseases.

**Prof. Dr. Harald zur Hausen,**  
**Nobel Prize Recipient in Medicine**  
**Board of Trustees, Care-for-Rare Foundation**



Research constantly seeks and delivers new knowledge. Knowledge that can cure rare diseases and alleviate suffering. The Care-for-Rare Foundation makes a significant contribution in this field, not just for children with rare diseases, but for the entire field of medicine!

**Professor Yu-Lung Lau MBChB, MD (Hon), FRCPC, FRCPS (Glas), FHKAM, FHKCPaed**  
**Chair Professor of Pediatrics**  
**LKS Faculty of Medicine, The University of Hong Kong**



The Care for Rare Foundation which has given tremendous assistance and support to improve care for children with rare diseases of the immune system in South-East Asia. With this partnership bond we are positive of a brighter future for patients with rare diseases. Another plus for humanity.

**Jonas Kaufmann, Opera singer**



With great pleasure and respect I support the mission of the care for rare Foundation. By caring for children with rare diseases, physicians and scientists around the world explore new frontiers, they are committed to help and to develop urgently needed cures. Musicians and artists can make a difference by building alliances to empower those, who need help to accomplish their mission.

**Karin Seehofer, Bavaria's First Lady and the wife of the Bavarian Minister-President**



The Care-for-Rare Foundation serves children from all over Bavaria and also from much further afield. I know that the doctors, nurses, carers, psychologists and all the other professional groups involved are tirelessly committed to helping children with rare diseases. We all need to support them in their efforts!

**Bruce Beutler M.D.,**  
**Nobel Prize Recipient in Medicine**  
**Board of Trustees, Care-for-Rare Foundation**



The destiny of children with rare diseases depends critically on enthusiastic scientists and international collaboration. The Care-for-Rare Foundation provides a valuable platform for both – therefore, I proudly share its vision and mission!

**Sandy Frankel, CEO Charitable Helmsley Trust, New York, USA**



The Helmsley Charitable Trust supports the Care-for-Rare Foundation as part of a worldwide initiative to help children with early onset Inflammatory Bowel Disease. Our funds both help children and their families who are in dire need, and may also unlock clues to the disease to help develop new treatments and a cure for everyone with IBD. Dr. Klein and his team are connected to medical centers across the world, bringing hope to many children and their parents.

**Prof. Dr. Wolfgang Heckl, Director of the Deutsches Museum**



Thanks to the latest technological advances, modern medicine is experiencing a dramatic transformation at many different levels. We should not forget the lot of sick children in this context; their diseases are so rare that mainstream research and the pharmaceutical industry are not interested in them. It is here that the Care-for-Rare Foundation provides their marvellous social mission – we need to support the Foundation in its commitment to the orphans of medicine!

**Ulrich Wilhelm, Director, Bavarian Broadcasting**



As a journalist, it is a subject very close to my heart to give a voice to every single young patient who barely enters public consciousness as an orphan of medicine. We must do our utmost to ensure that the dignity of every child is respected!

**Dr. Stefan Leifert, German Television Broadcasting**



The Care-for-Rare Foundation is an impressive organisation with its mixture of medical excellence and tireless commitment. Its network connects doctors and scientists all over the world in order to give a future to children with rare diseases.

**Dr. Nikolaus von Bomhard, Chairman of the Executive Board, Munich Re Group**



In the Care-for-Rare Center at the Dr. von Hauner Children's Hospital, research and practice work hand in hand in terms of the patients' treatment, particularly in the case of rare diseases, which is of great benefit to the children.

**Her Royal Highness Princess Ursula of Bavaria**



The Care-for-Rare Foundation sets a shining example; the Foundation refuses to settle on the idea that children with rare diseases have no hope of a cure. It is committed to giving hope to these children through science and research and does this with enthusiasm and a high level of expertise. The Care-for-Rare Foundation needs our support, let's help them!

**Dr. Paul Achleitner, Chairman of the Board of Directors, Deutsche Bank AG**



The Care-for-Rare Foundation takes up the forgotten fate of children with rare diseases. Renowned pioneers of medicine and science are committed to promoting research and thus providing sustainable help for children all over the world. Within a very short space of time, the Foundation has managed to rise and stand among the most effective charitable Foundations in Germany, Care-for-Rare deserves our support!

**Nina Ruge, Presenter and journalist**



Care-for-Rare has won me over – I greatly admire their commitment, their expertise and their enthusiasm. Time and again Care-for-Rare has shown that we can help children with rare diseases through our combined support!

**Marc B. Wolpow,**  
Co-Chief Executive Officer, Audax Group,  
Boston, USA



The Care-for-Rare foundation is a unique philanthropic entity. Established by visionary scientists and empowered by a growing network of private and corporate institutions, the Foundation's team truly make a difference for children with rare diseases. I am impressed how fast the Foundation has been able to assemble the world's best clinicians and scientists devoted to unravel the mysteries of rare diseases and to jointly develop urgently needed new therapies!

**Michael Apkon, MBA, MD, PhD**  
President and CEO at The Hospital for Sick  
Children, Toronto



The Hospital for Sick Children, SickKids, at the University of Toronto is Canada's premier academic institution for pediatric health care. We have a long and successful history of striving towards excellence in the best interest of the children of our society. The international Care-for-Rare Foundation with its headquarter in Munich, Germany, is an excellent example for growing interdisciplinary and international collaborations. SickKids is a partner of the Care-for-Rare Alliance, together we are committed to decipher the genetics of early onset inflammatory bowel diseases and to design innovative treatment strategies.

**Prof. Dr. Joseph Penninger**  
Director, Institute for Molecular  
Biotechnology, Vienna  
*Board of Trustees, Care-for-Rare Foundation*



The Care-for-Rare Foundation supports top researchers around the world. This phenomenal Foundation with its unique spirit and enthusiasm is of fundamental importance in finding treatments for life-threatening diseases!

**Vicki and Fred Modell,**  
Jeffrey Modell Foundation NY



The tragic loss of our son Jeffrey has prompted us to change the destiny of children with primary immunodeficiency diseases around the world – the international care-for-rare Foundation is a wonderful partner for this mission and Christoph Klein as spiritus rector together with his outstanding team has made remarkable progress.

**Professor Aziz Bousfiha, Professor of Pediatrics, Casablanca, Chair Pediatrics and African Association of Immunodeficiencies**



With great level of enthusiasms I am a sincere well-wisher of the Care-for-Rare Foundation for children with rare diseases. The African continent is part of our shared world but its children are often bound to suffer because access to preventive and curative medical measures are limited. The Care-For-Rare Foundation partners with us providing resources for education and research. Even though this support is still limited at this time, I am very optimistic that the spirit of collegiality, enthusiasm and optimism across nations and continents will eventually translate into a better health care system and a brighter future for those who are greatly disadvantaged today. The Care-for-Rare Foundation is a paradigm for these efforts.

**Prof. Dr. Heribert Prantl, Member of the Main Editorial Office, Süddeutsche Zeitung**



Every child has the right to expect health, protection and care from the community. Every sick child, above all, needs help in accordance with the latest developments of medical science. There are shortcomings even here in Germany; this must not deter us. We must recognize and put an end to these failings; we owe that to the children. The wellbeing of children is not a luxury. It is the fundamental right of every child and the fundamental duty of our society.

**Professor Kanya Suphapeetiporn MD PhD,**  
Chulalongkorn University, Bangkok



Partnering with the international Care-for-Rare Foundation, we promote public awareness for rare diseases and enjoy being a part of an even larger network dedicated to train future leaders in pediatrics. Care-for-Rare has had a wonderful impact on how we currently rethink our responsibility for sick children with rare diseases and on how we will direct future research studies aiming to cure them.

**Felix's Family**



The doctors at the Care-for-Rare Foundation helped us a great deal. At the beginning of our fight with the illness, they made it possible to reach a very rapid diagnosis; they never gave up and they always encouraged us when we felt desperate. Now Felix has got over his illness and is healthy again. We are very grateful for all the things that the Foundation made possible!

**David G. Nathan MD**

**Robert A. Stranahan Distinguished Professor of Pediatrics and Professor of Medicine, Harvard Medical School, em. Physician-in-Chief, Boston Children's Hospital**



The international Care-for-Rare Foundation for children with rare diseases is a remarkable initiative with a truly humanitarian vision: no child should be destined to die because of a rare disease – regardless of national and ethnic origin and regardless of the financial situation of his or her parents. This mission is perfectly in line with Boston Children's Hospital mission „until every child is well“!

We are proud to be part of the international Care-for-Rare Alliance.







## How you can make your contribution

To put its vision into practice, the Care-for-Rare Foundation is dependent on personal and financial support. Your volunteer efforts are appreciated as well as your financial support. You can help us by making your personal network of friends and acquaintances aware of the global commitment of the Care-for-Rare Foundation. By doing this, you will support an important aim of the Foundation, because a great number of people still do not know much about the concerns and needs of children with rare diseases. In some circumstances, you may be able to place your talents at the service of the Care-for-Rare Foundation, just as some consultancy and media agencies, and various creative artists and experts in the fields of law, finance and the media already do. We are making an effort to expand this alliance further and very much appreciate it when people volunteer their time. You can also give us financial assistance.

The following section, shows you some options of how you can support us and illustrates these with specific examples; however, you are free to be as creative as you wish!

There are numerous different ways to support the mission of the Care-for-Rare Foundation. We would be happy to develop a strategy for your charitable contribution tailored to your individual circumstances.

### Private donations

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Have you already satisfied your basic material needs and would therefore prefer to celebrate your anniversary or a personal event with a donation to a good cause?

In that case, we invite you to ask your guests to make a donation to the Care-for-Rare Foundation. We will use these resources in the near future to realize the aims set out in our statutory objectives. Each donation is spent directly, with no deductions made, on one of our projects. If requested, we will, of course, issue you and



your guests with a donation receipt which can be used for tax purposes. With your donation, whether large or small, you will take part in the life-saving treatment of a child with a rare disease. You enable us to pay the travel costs or the necessary medication, in the case that the state social security system does not cover these costs.

Example: *Jörg Richter, a passionate cyclist, fulfilled his dream in the summer of 2015 by cycling right across the United States from Seattle to New York. He organized his 100-day tour under the motto of the Care-for-Rare Foundation "From Discovery to Cure" and collected donations for the Care-for-Rare Foundation on his 6,000 km long cycling trip.*

### Corporate donations

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Would you and your company like to set an example for social commitment and back your sense of social responsibility with actions? Perhaps you might think at first glance that the Foundation's goals have little to do with the spirit of your company; we would be happy to



help you find similarities, so that your company, too, can make a sustainable social contribution towards shaping the future of our society, which is at the same time, closely aligned with your corporate goals and values.

*Example: Several pharmaceutical companies have joined together to form the “Club of 50” in order to ensure the training of physician-scientists in the pediatric sub-disciplines. This commitment has made it possible to strengthen the level of care for chronically ill children – and guarantee a future of further high-quality advances in medicine. Other companies donate a share of their specialised expertise to us and in this way help the Care-for-Rare Foundation attract further networks and constantly continue to develop the Foundation’s work.*

### **Trust loans**

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Are you unable to predict at this point whether you will need to use your assets to ensure the financial wellbeing of your family in the future? In this case, you can grant the Care-for-Rare Foundation a donation for a limited

period of time in the form of a trust loan. As a lender, you can make a contractual agreement with us to set the amount of the trust loan, the term and the period of notice. We would be happy to assist you in this. The tax-free investment income flows into the Care-for-Rare Foundation and is used to accomplish the goals set out in its statutory objectives.

*Example: With a trust loan of 100,000 euros on a temporary basis, you will give access to a genome-wide diagnostic examination to up to five children a year for the targeted diagnosis of their rare diseases and in addition, pave the way for the development of new therapeutic interventions.*

### **Endowments**

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Have you inherited money or have other financial resources at your disposal which you would like to put to work for a good cause in your name? Education programs can be set up in your name?

Further education programmes can be set up in your name if you so wish using the income from your endowment.

*Example: In this way, we have funded a doctor’s post in the field of pediatric cancer research in the memory of a manager who died at an early age. As the result of a new endowment, we have been able to set up the Daniel Schilo-Silberman Fellowship and thus give time for reflection and promote creative, unconventional science, so that children with cancer can be cured more effectively in the future.*

### **Trust foundations**

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If you are considering setting up a foundation, but are balking at the administrative burden and the continuous commitment required, you can set up a trust foundation quickly and without great cost under the umbrella of the Care-for-Rare Foundation.

*Example: By setting up of a trust foundation, you give the Care-for-Rare Foundation the possibility of continuously providing training for young doctors and scientists from all over the world. Following the princip-*

le of “Train-the-Trainer”, the sphere of influence of your commitment increases because we fund the most talented young scientists and thus open new horizons for them for their respective place of work, be it in Europe, South America, the Middle East or East Asia. Young people from less privileged regions sponsored in this manner are shaped in many ways and thus become ambassadors of international understanding.

## Will

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Would you consider creating a lasting legacy? A financial gift made in your name to ensure our mission for the lives of children in future generations?

You can give a strong and sustainable indication of support for children with rare diseases by keeping us in mind as the recipient of a part of your assets. You can thus ensure that your name and legacy live on.

With a group of experts at our disposal, we can assist you free of charge and without obligation with any questions you may have about drawing up your will.

You can contact us as +49 (0) 4400 5 7700. Call us for a non-binding consultation.

Example: *From the income from your legacy, you can guarantee, for example, the continuation and intensification of the guiding light provided by the Care-for-Rare Alliance in the Dr. von Hauner Children’s Hospital at the LMU. You would help us to fund excellent science to the benefit of sick children, to give that little extra that state institutions are no longer able to provide due to their minimal standards limitations. This includes not only laboratory furnishings and equipment, but also the co-financing of our so-called core units, the shared technology platforms (e.g. genomic diagnostics) or the interdisciplinary pediatric study centre. Of particular relevance, your legacy can help ensure the realization of our Care-for-Rare Translational Research Centre next to new main building of the Hauner Children’s Hospital.*

By becoming a charitable donor to the Care-for-Rare Foundation, you will have our heartfelt gratitude. In accordance with your personal preferences, we can either praise your generosity in public or treat it with complete discretion. You can receive regular information about the ongoing development of the Foundation and reports on project successes. You can be immortalized by having your name inscribed on our funder wall or in certain circumstances, having a fellowship project or room named in your honor. In addition you will receive exclusive invitations to special events.

**We look forward to getting in touch with you!**

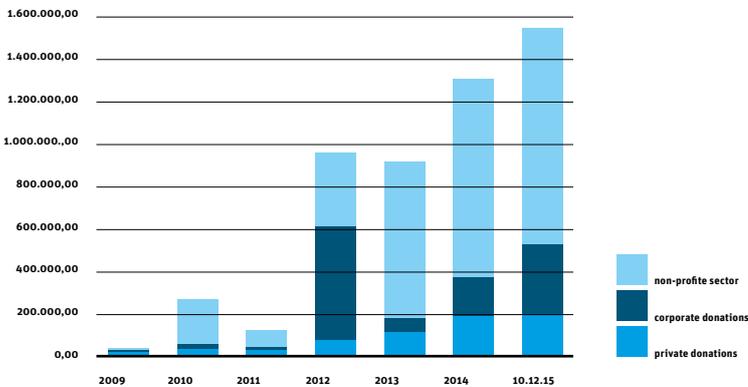
A new Dr. von Hauner Children’s Hospital will be built by the Bavarian Government within the next few years. We are now searching for supporters to realize a dedicated Care-for-Rare Translational Research Center, as computer-visualized by the Architects Nickl & Partners (left: New Dr. von Hauner Children’s Hospital, right: Care-for-Rare Translational Research Center).



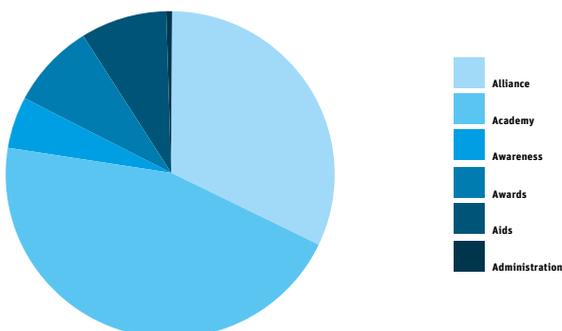
### Care-for-Rare Foundation organizational chart



### Annual donations to the Care-for-Rare Foundation (in €)



### Distribution of sponsorship according to funding sectors



### Board of Directors

Christoph Klein, MD PhD, is Director of the Dr. von Hauner Children's Hospital at the Ludwig-Maximilians-Universität München, where he oversees an extensive clinical and research portfolio devoted to preventing, diagnosing, and treating children with rare and common diseases.

He is the founding spokesman of the German research networks on rare diseases and also serves as counselor to the Ministry of Research and Education and the Ministry of Health on issues related to rare diseases as well as initiatives to bolster medical health measures for sick children. He is the co-founder and principal architect of the international Care-for-Rare Foundation.

Upon subspecialty training in pediatric immunology (Hôpital Necker Enfants Malades, Paris) and pediatric hematology/oncology (Boston Children's Hospital, Harvard Medical School) he held faculty appointments at Harvard Medical School and Hannover Medical School before being nominated chairman of the Department of Pediatrics and Adolescent Medicine at LMU. Dr. Klein has made seminal contributions to the understanding of how blood and immune cells develop and control immunity and tolerance. Together with his team, he has identified and characterized numerous inherited diseases of the immune system and developed novel cell- and gene-based therapies. Dr. Klein is a member many scientific societies and is the recipient of numerous prestigious awards including the Adalbert Czerny Prize, the Hector Fellow Prize, the William-Dameshek Prize and the Gottfried Wilhelm Leibniz Prize, the most prestigious science award in Germany.

Professor Dr. Andreas Staudacher is a certified specialist in administrative law and heads a nationwide office. As Professor of Administrative and Construction Law, he lectures at the Universities of Biberach and Saarbrücken.

## Scientific Advisory Board

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Professor Jean-Laurent Casanova MD PhD  
*Professor at St. Gildes Laboratory of Human Genetics of Infectious Diseases at Rockefeller-University, New York. Professor Casanova investigates the genetic causes of infectious diseases.*

Professor Dr. rer. nat. Ulrike Gaul  
*Humboldt Professor at the Gene Centre at Ludwig Maximilians Universität, Munich. Professor Gaul is a developmental biologist specializing in the field of neurobiology and systems biology.*

Professor Scott Snapper MD PhD  
*Professor of Gastroenterology, Children's Hospital Boston, Harvard Medical School. Professor Snapper investigates the mechanisms of immunity and tolerance in the intestinal tract.*

## Board of Trustees

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Prof. Dr. Bruce Beutler,  
*2011 Nobel Laureate in Medicine*  
Dr. Thomas Jaschke  
Dr. Martin Ney, *Ambassador of the Federal Republic of Germany in India*  
Prof. Dr. Joseph Penninger, *Director of IMBA Vienna*  
Prof. Dr. Joseph Sayer, *former Misereor CEO*  
Hubert Thaler,  
*Board of Werner Reichenberger Foundation*  
Prof. Dr. Harald zur Hausen,  
*2008 Nobel Laureate in Medicine*

## Patroness

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Annette Schavan, *Ambassador of the Federal Republic of Germany to the Holy See*

## Foundation Council

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Dr. rer. pol. Thomas Jaschke studied Business Management at the University of Muenster and afterwards took on a variety of international executive roles with the Bertelsmann Group. He saw through the setting up of the Care-for-Rare Foundation as Chairman of the Board of Trustees of the Foundation and is now developing its subsequent strategic orientation as a member of the Foundation Council.

## Good Practice for Foundations

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The Care-for-Rare Foundation is under the obligation to comply with the principles of good practice for foundations and supports the initiative for a transparent civil society. We are happy to send you our statutes and rules of procedure upon request. The Care-for-Rare Foundation for Children with Rare Diseases is a legal Foundation recognized as a charity under private law. The members of the Board of Directors, the Board of Trustees and the Foundation Council are active in an honorary capacity. All donations are spent on project work without any deductions; administrative costs are covered by defined overhead rates for each project provided by the main sponsor

## Awards

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The Care-for-Rare Foundation was assessed as excellent by the "Germany – Land of Ideas" initiative (2009). The social commitment of the Foundation was awarded the Cusanus Prize (2015). Furthermore, the Care-for-Rare Foundation has been nominated for the Avicenna Prize (2016).



## **Acknowledgements**

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We are grateful to all friends and supporters who show enthusiasm for the mission of the Care-for-Rare Foundation and regularly help us with small and large donations alike. It is only through this generous commitment of citizens that it is possible for the Foundation to grow and to alleviate the suffering of children with rare diseases.

The Care-for-Rare Foundation is also supported by a growing network of professionals in the fields of medicine and natural sciences, economics and law, design and marketing, art and culture, management, journalism and politics. They share their expertise and advice with us.

We would like to thank our charitable supporters, in particular Sternstunden e.V., Reinhard Frank Foundation, Werner Reichenberger Foundation, Tribute to Bambi Foundation, Helmsley Charitable Trust NY, USA, Cyliax Foundation, Jeffrey Modell Foundation NY, USA, Hörer helfen Kindern e.V., Radio ffn, SZ Adventskalender.

We would also like to acknowledge great support by our corporate supporters, especially all members of the "Club of 50" (Alexion Pharma Germany GmbH, Bayer Pharma AG, bene Arzneimittel, Boehringer Ingelheim GmbH, Celgene GmbH, CSL Behring GmbH, MSD Sharp Dohme GmbH, Roche, Shire Deutschland GmbH, sigma-tau Arzneimittel GmbH, Wacker Chemie AG), Gmund Papier, HypoVer-einsbank, V-Bank, Munich Airport, Sausalitos Holding, AOK Bayern BAIN & Company, eberhardwolf.com, BMW Munich, LEGO, Hilton International, Hirmer, HIPPI, Steiff and many others.

We are very grateful to their help and engagement!

## **Foundation Supervisory Authority**

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The Care-for-Rare Foundation® is recognized as a charity and legal entity according to German Civil Law.

Supervisory Authority:  
Regierungspräsidium Tübingen  
Konrad-Adenauer Strasse 20  
D-72072 Tübingen

Tax ID Number Germany: 88041/00409  
For US Non Profit 501(c) 3 tax exempt details  
please refer to the Care-for-Rare Website

## **Address**

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## **Bank Account**

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There are many ways of supporting the Care-for-Rare Foundation. The most important thing for us is to have a personal discussion with you. Together we can decide how your wishes can be realized to best effect.

Please call us!  
My personal telephone number is  
+49 89 4400 5 7700.

No child should have to die from a rare disease –  
Help us turn this into reality step by step!



**Care-for-Rare Foundation**

Foundation for Children with Rare Diseases

[www.care-for-rare.org](http://www.care-for-rare.org)