# Rapport Annuel

# *Annual Report*

# 2016

BLACKSWAN®
FOUNDATION

SWISS FOUNDATION FOR RESEARCH ON ORPHAN DISEASE S
SCHWEIZERISCHE STIFTUNG FÜR DIE FORSCHUNG SE LTENER KRANKHEITEN
FONDATION SUISSE POUR LA RECHERCHE SUR LES MALADIES ORPHELINE S
FONDAZIONE SVIZZERA PER LA RICERCA SULLE MALA TTIE ORFANE

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# MESSAGE DU PRÉSIDENT

L'année 2016 a été une année importante pour la Fondation BLACKSWAN et pour le milieu scientifique. En effet, à la Fondation BLACKSWAN, nous croyons qu'un engagement plus fort au niveau international est fondamental pour attirer plus de ressources et développer des thérapies pour des millions de patients. C'est pourquoi, en 2016, la Fondation a lancé le programme #RAREvolution. L'un des principaux objectifs de la #RAREvolution est d'améliorer la compréhension des RD parmi le public, les décideurs, les professionnels de la santé, les chercheurs et les universitaires avec de nouvelles interventions visant à intégrer son action internationale de plaidoyer et de sensibilisation.

L'initiative RE(ACT), quant à elle, fait partie du programme #RAREvolution et favorise la coopération internationale dans la recherche scientifique sur les RD. Les efforts de recherche existants sont encore trop éparpillés et les recherches fragmentées sont menées avec peu de coordination entre les laboratoires de recherche. Ce manque de coordination est particulièrement préjudiciable à l'accroissement des connaissances sur les RD, en particulier dans ce domaine où les ressources sont très limitées et la population de patients est faible. Par le biais de l'initiative RE(ACT), la Fondation BLACKSWAN promeut la création d'une communauté de chercheurs afin de montrer aux institutions publiques les importants résultats obtenus et d'acquérir une influence politique plus forte pour demander plus de soutien.

Nous avons tenu la troisième édition du Congrès RE(ACT) 2016 à Barcelone, Espagne. Le congrès a été organisé par la Fondation BLACKSWAN et E-RARE, le réseau ERA-Net pour les programmes de recherche sur les maladies rares. L'événement a été un grand succès et a confirmé la nécessité des deux points développés précédemment, soit l'amélioration des collaborations internationales et le besoin de créer un réseau solide entre les chercheurs et toutes les parties prenantes et donc de la légitimité de l'initiative RE(ACT) et du programme RAREvolution. Lors du Congrès RE(ACT), la Fondation BLACKSWAN a également lancé une pétition en ligne contenant neuf recommandations pour guider l'élaboration de politiques sur la recherche sur les maladies rares.

Je suis fermement convaincu que la collaboration de toutes les parties prenantes pour montrer l'importance des maladies rares dans le programme de santé permettra aux organisations internationales, telles que l'OMS et d'autres organismes des Nations Unies, de reconnaître les maladies rares comme une priorité en matière de santé publique et de recherche et de sauver la vie de millions d'individus.

Olivier Menzel
Président et fondateur

***"La Fondation BLACKSWAN facilite la collaboration au sein de la communauté des maladies rares"***

# *MESSAGE FROM THE PRESIDENT*

*2016 is an important year for the BLACKSWAN Foundation and for the research community to advocate for more research on rare diseases (RDs).*

*At the BLACKSWAN Foundation we believe that a stronger engagement at international level is fundamental to attract more resources, create new incentives for research and develop therapies for millions of patients. For this reason, in 2016 the Foundation started the #RAREvolution program that combines the work and experience developed in the last years through the RE(ACT) Initiative, (RE(ACT) Congress and RE(ACT) Community) with new interventions aimed at integrating its international advocacy and awareness action.*

*One of the main objectives of the #RAREvolution is to improve the understanding of RDs among the public, policy-makers, health professionals, researchers and academics,.*

*The RE(ACT) Initiative is part of the #RAREvolution Program and promotes international cooperation in scientific research on RDs. Existing research efforts are in fact still scattered, and fragmented research is being performed with little coordination between research laboratories. This lack of coordination is particularly detrimental to the increase of knowledge on RDs especially in this field where resources are very limited and the patient population is small.*

*Through the RE(ACT) Initiative, the BLACKSWAN Foundation promotes the creation of a community of researchers, which reveals the needs of this field, shows to public institutions the important results achieved and gain stronger political leverage to ask for more support.*

*We held the third edition of the RE(ACT) Congress 2016 in Barcelona, Spain. The Congress was organized by the BLACKSWAN Foundation and E-RARE, the ERA-Net for Research Programs on RDs. and was a great success in improving international collaborations and confirmed the ultimate need of creating a strong network amongst researchers and all stakeholders.*

*During the RE(ACT) Congress, the BLACKSWAN Foundation launched an online petition providing nine recommendations as guidance for the establishment of policies on rare diseases research.*

*I strongly believe that the collaboration of all stakeholders in showing the importance of rare diseases on the health agenda will bring international organizations, such as the WHO and other United Nations agencies, to recognize rare diseases as a public health and research priority and save the life of millions of individuals.*

*Olivier Menzel*
*Chairman and Founder*

***"The BLACKSWAN Foundation facilitates the collaboration within the rare disease community"***

# QU'EST-CE QU'UNE MALADIE RARE OU ORPHELINE

En Suisse et dans l'Union européenne, une maladie est dite rare lorsque moins d'une personne sur 2'000 est atteinte, que la maladie est chronique et incapacitante et qu'un effort particulier doit être fourni pour développer un traitement. L'on retient aussi que les maladies rares réduisent souvent l'espérance de vie de qui en souffre.

Au niveau international, il n'y a pas de consensus autour du seuil d'incidence retenu pour définir la rareté. Aux États-Unis par exemple, il s'agit de toute pathologie ou condition de santé affectant moins d'une personne sur 1'500 (soit 200'000 personnes sur le territoire). L'on désigne par ailleurs une maladie comme orpheline lorsqu'il n'existe aucun traitement pour en venir à bout. Les médecins ne connaissent pas l'affection, la recherche et le diagnostic sont inexistants et les victimes, totalement délaissées du système sanitaire, souffrent d'un manque de reconnaissance quasi total.

Os de verre, syndrome du cri du chat, syndrome de Lowe… Les maladies rares et orphelines recouvrent une réalité très diversifiée. Elles peuvent empêcher de bouger (myopathies), de voir (rétinites), de comprendre (X fragile), de respirer (mucoviscidose), de résister aux infections (déficits immunitaires) etc. On en dénombre à ce jour au moins 8'000 dont le 80% au moins a une origine génétique. Il s'agit de pathologies infectieuses ou auto-immunes dans le 20% restant

## UN PARCOURS DU COMBATTANT

Les enfants sont particulièrement concernés : trois malades sur quatre ont moins de dix-huit ans, et les maladies rares sont spécialement mortelles et incapacitantes dans leur cas. Ce alors que le corps médical peine souvent à établir un diagnostic de par le faible nombre de personnes touchées.
Outre la difficulté à se faire reconnaître et une espérance de vie réduite, les malades souffrent de handicaps à l'origine de discrimination et d'isolement. Il existe heureusement des associations pour leur venir en aide ainsi qu'à leurs proches.

# WHAT IS A RARE OR ORPHAN DISEASE ?

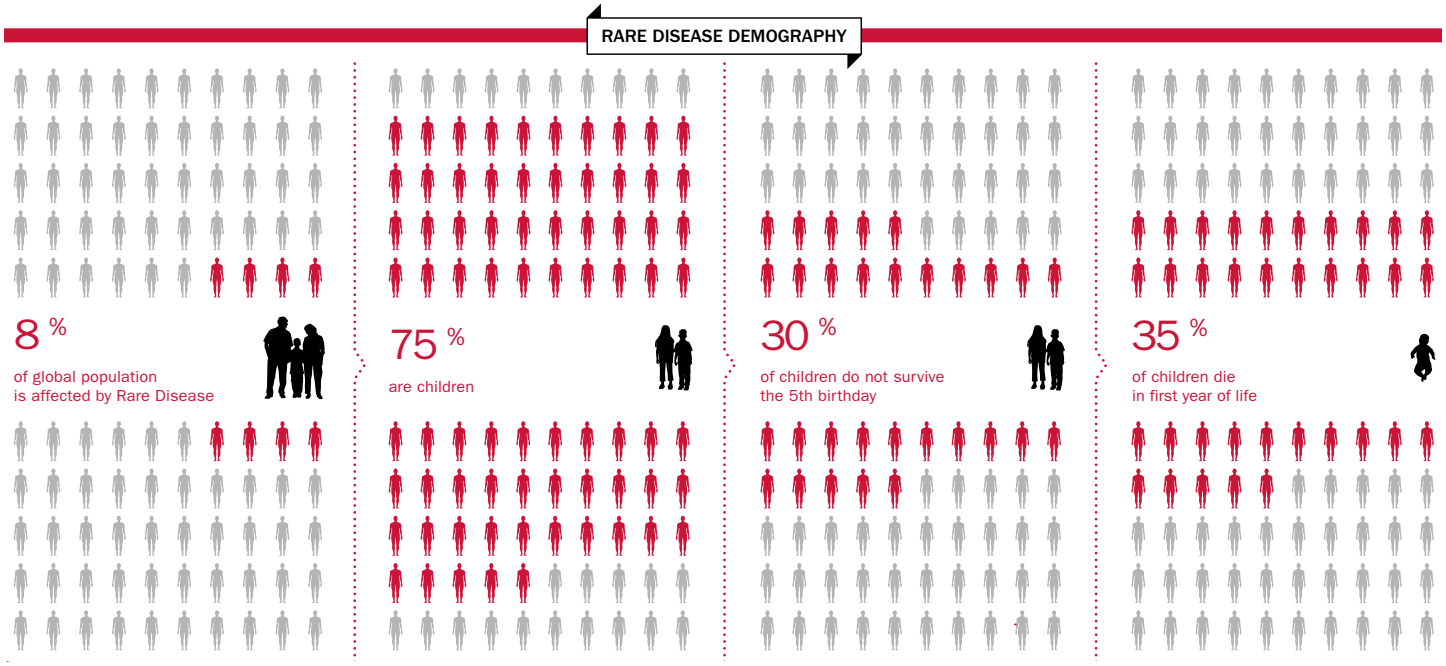
*A disease or disorder is defined as rare in Europe and in Switzerland when it affects not more than 1 in every 2,000 persons. These conditions are generally chronic, progressive, debilitating and often life-threatening. In addition, these rare diseases present certain challenges for the development of new treatments.*

*There is no internationally accepted definition of rare diseases. For example, in the United States, a condition is rare when affects 1 in every 1,500 persons (or less than 200,000 citizens). Furthermore, it is considered orphan any rare disease for which no treatment has been developed. Practitioners do not know the condition, research is not ongoing and it is extremely difficult to receive a diagnosis for lack of scientific knowledge. For these reasons patients feel abandoned by the health system and suffer from an almost total lack of care.*

*Osteogenesis imperfecta, Lowe syndrome, cri-du-chat syndrome…rare and orphan diseases are a very diverse reality. These may affect one's ability to move (myopathies), to comprehend the world around us (fragile X syndrome), to breathe (cystic fibrosis), or to combat infection (immune deficiencies). It is estimated that there are at least 8,000 orphan diseases today, 80 per cent of them are genetic in origin. The remaining 20 per cent are autoimmune and infectious disorders.*

### *A REAL STRUGGLE*

*Children are particularly concerned by rare diseases: three out of four patients are under the age of eighteen and in their case rare diseases are particularly life threatening and disabling and medical professionals struggle to make a diagnosis because of the small number of people affected.*
*In addition to the difficulty in obtaining recognition and to a reduced life expectancy, patients suffer from disabilities that lead to discrimination and isolation. Fortunately, there are associations that help them, their families and loved ones.*



# LA RECHERCHE SUR LES MALADIES RARES SE HEURTE À PLUSIEURS DIFFICULTÉS MAJEURES

> Des obstacles d'ordre scientifique : le faible nombre de malades recensés ne permet souvent pas de valider scientifiquement les résultats du travail de recherche, ce qui empêche la publication d'articles qui pourraient mobiliser l'attention et l'intérêt de la communauté des chercheurs;

> Des obstacles d'ordre financier : les agences nationales de financement public ne sont pas proactives dans le soutien de la recherche à cause du manque d'expertise des commissions internationales d'évaluation des projets et du nombre restreint de publications scientifiques de la part des chercheurs demandant un financement.

> Des obstacles d'ordre commercial: ces pathologies touchant un nombre très restreint de personnes, les grandes firmes pharmaceutiques négligent d'investir des moyens dans le développement de médicaments.

La recherche sur les maladies rares et orphelines présente pourtant un intérêt majeur, car elles peuvent servir de modèles pour des affections plus communes et aider à développer des médicaments efficaces à plus large échelle.

On l'a vu dans le cas de la leucémie, maladie qui est devenue chronique plutôt que mortelle grâce au développement par une grande firme pharmaceutique d'un médicament conçu à la base pour soigner une forme de leucémie rare des enfants, réfractaire aux médicaments à l'époque sur le marché. Par ailleurs, certaines start-ups ou entreprises se sont spécialisées dans la vente de médicaments pour les maladies rares et leur business model, bien que marginal, a prouvé qu'il fonctionnait.

Pour relever le défi de la recherche, il est cependant nécessaire que les spécialistes puissent échanger autour de compétences pluridisciplinaires et de bonnes pratiques, et que le plus grand nombre de patients participent à la recherche. Il s'agit aussi de renforcer les liens entre le monde académique et l'industrie pour que celle-ci puisse concrétiser les résultats obtenus en nouveaux outils diagnostiques et thérapeutiques.

# *RESEARCH ON RARE DISEASES FACES SEVERAL MAJOR CHALLENGES*

*> Scientific obstacles: the small number of patients often does not allow to scientifically validate the results of the research work, which prevents the publication of articles that could mobilize the attention and interest of the research community;*

*> Financial barriers: National public funding agencies are not proactive in supporting research because of the lack of expertise of international project assessment boards and of the limited number of scientific publications by researchers requesting funding.*

*> Commercial obstacles: these diseases affecting a very limited number of people, are often neglected by large pharmaceutical companies that are reticent to invest resources in the development of medicines. Research on rare and orphan diseases is of considerable importance as it can serve as a model for more common diseases and help develop effective drugs on a larger scale.*

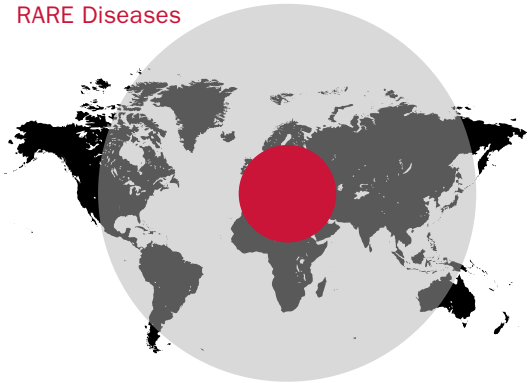
*As has been seen in the case of leukemia, when a large pharmaceutical company developed a drug designed at the base to cure a rare pediatric form of leukemia that was resistant to the drugs available at the time on the market. This drug then revealed to be effective at fighting off all forms of leukemia.*
*In addition, in recent years, some start-ups and companies specialized in the development of drugs for rare diseases, and their business model proved to be effective.*

*In order to meet the challenge of research, however, it is necessary for specialists to exchange best practices and use collaborative models and that the largest number of patients participate in research. There is a need to strengthening the partnerships between the academic world and the industry to obtain new diagnostic and therapeutic tools.*

**RARE DISEASES GLOBAL IMPACT**

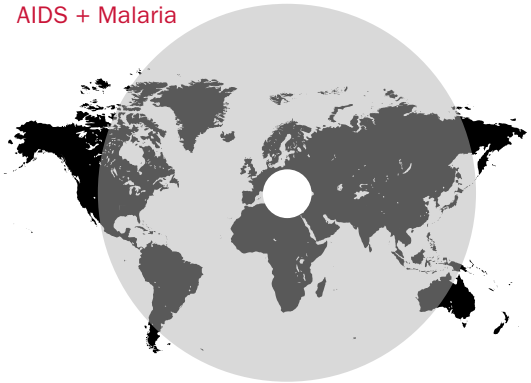
475 MIO/7'052 MIO

RARE Diseases



228 MIO/7'052 MIO

AIDS + Malaria



# LA FONDATION

## LA FONDATION EN BREF

Les maladies rares affectent 6 à 8% de la population mondiale et quelque 500'000 personnes en Suisse. Lorsqu'il s'agit d'enfants – dans trois cas sur quatre – l'issue est très souvent fatale. La quête de médicaments adaptés est donc une priorité, d'autant que leur efficacité a été démontrée dans le traitement de pathologies plus fréquentes.

La Fondation BLACKSWAN a été créée en Suisse en 2010 pour aider partout dans le monde celles et ceux qui sont impliqués dans ce combat. Elle soutient la recherche sur tout type de maladie rare ou orpheline, ce qui la rend unique au monde.

Outre son travail dans la quête de fonds, elle organise chaque deux ans un congrès appelé RE(ACT) Congress, devenu une référence internationale. En trois éditions, plus de 1500 scientifiques ont pu mettre en place une cinquantaine de collaborations.

La RE(ACT) Community est quant à elle à la fois un outil de financement participatif (crowdfunding) et une plateforme d'échanges autour de cette problématique. Enfin, la Fondation mène depuis 2015 une campagne appelée #RAREvolution afin que les maladies rares deviennent une priorité de la recherche et de la santé publique.

# THE FOUNDATION

## THE FOUNDATION IN BRIEF

Rare diseases affect 6% to 8% of the world population and approximately 500,000 people in Switzerland. The majority of these conditions affect children and in three out of four cases have a fatal outcome. The quest for specific drugs is a priority, especially as the effectiveness of certain rare disease drugs has been demonstrated in the treatment of most common diseases.

The BLACKSWAN Foundation was established in Switzerland in 2010 to contribute to the development of research on rare and orphan diseases worldwide. The Foundation supports research on all different types of rare and orphan disease, which makes its vision unique and helps in finding new solutions that can assist a large variety of projects.

Besides its funding research mission, the Foundation organizes every two years the RE(ACT) Congress, which became an international reference point for rare disease experts. In three editions, more than 1,500 scientists were able to establish approximately fifty collaborations.

In 2014, the BLACKSWAN Foundation launched the RE(ACT) Community a crowdfunding and a knowledge sharing digital platform that connects researchers, patients and other rare disease stakeholders. Since 2015, BLACKSWAN Foundation has also started an international advocacy and awareness campaign called #RAREvolution to ensure rare diseases are recognized as an international public health and research priority.

1 338 612 968 CHINA POPULATION

1 156 897 766 INDIA POPULATION

475 304 355 RARE DISEASE POPULATION

302 074 000 USA POPULATION

# 2016 ACTIVITIES
# SUPPORT FOR SCIENTIFIC RESEARCH

In 2016, BLACKSWAN Foundation efforts to support scientific research focused on the consolidation of the online platform, the RE(ACT) Community. The community is conceived to help researchers working in the field of rare and orphan diseases to create new collaborations, share knowledge and promote their scientific projects.

The main objective of the RE(ACT) Community is to increase international cooperation on rare disease's research worldwide and thus accelerating the delivery on the market of new molecules and therapies for millions of patients.

The Community also encourages the creation of a society of researchers that can raise awareness about the needs of this sector, emphasizes the results achieved to gaining stronger political leverage and ask for more support from public institutions.

The online platform is organized around four main axes: Meet, Learn, Share and Support. Meet other researchers and find new collaborations; Learn from the knowledge and experience of other researchers and patients; Share scientific knowledge and facilitate the exchange of information between researchers and patients. It also offers the possibility to financially support research projects by starting a crowdfunding campaign on its website.

# 2016 ACTIVITIES
# SUPPORT FOR SCIENTIFIC RESEARCH

## THE RE(ACT) CONGRESS 2016 – INTERNATIONAL CONGRESS OF RESEARCH ON RARE AND ORPHAN DISEASES

The third edition of the RE(ACT) Congress — International Congress on Research of Rare and Orphan Diseases, organized by the BLACKSWAN Foundation (http://www.blackswanfoundation.ch/en/) in collaboration with E-Rare (http://www.erare.eu/) has been held at the Crowne Plaza in Barcelona, from 9th to 12th March 2016. The Congress supported by IRDiRC, Eurordis and CIBERER presented some of the most innovative and outstanding research on rare diseases on topics like drug repositioning and personalized medicine, NGS, pathophysiology and gene therapies.

The event involved more than 200 attendees: researchers, doctors, organizations, patients, sponsors and other international stakeholders. World-class speakers like Alex MacKenzie, Gert Matthijs, Olaf Horst Riess, Danilo Tagle and many others, shared their studies during the four days conference and were the protagonists of a successful edition of the RE(ACT) Congress 2016. The full list of the speakers is available on: http://www.react-congress.org/speakers/.
Speaking at the opening ceremony, Christopher P. Austin, Director of the National Center for Advancing Translational Sciences (NCATS) at the U.S. National Institutes of Health (NIH) underlined the importance of collaboration in rare diseases to catalyze the innovative methods and technologies for the development, testing and implementation of diagnostics and therapeutics.
Patients were represented at the Congress by the presence of Yann Le Cam, Chief Executive Officer of the European Organization for Rare Diseases – EURORDIS, who stressed the importance of building bridges between patients and the scientific community and as part of this objective announced a new collaboration with the BLACKSWAN Foundation.

The RE(ACT) Congress gathered not only the attendees interest, but also a wide online popularity. In fact, more than 350 contents were shared on the social networks with the official hashtags #REACTCongress, #RAREvolution, #REACTCommunity.
The RE(ACT)Congress 2016 has been chosen by the BLACKSWAN Foundation for launching the restyling of the RE(ACT) Community, (http://react-community.org/), the digital platform created in 2014. The graphic restyling went hand in hand with the definition of its overall mission, summarized in the claim: “We are the RAREvolutionary people. Stand up for Scientific Research”. The idea behind the Community is to provide a space where researchers and patient can learn from each other and support rare diseases projects through crowdfunding. The goal is to improve knowledge and involve different types of stakeholders in campaigns and concrete actions.

# 2016 ACTIVITIES
# SUPPORT FOR SCIENTIFIC RESEARCH

## ONLINE PETITION IN SUPPORT OF RARE DISEASES RESEARCH

Help us to increase support for rare diseases research and save millions of people

During the RE(ACT) Congress 2016, the Foundation launched an online petition on Change.org to provide guidance to policy makers on rare disease research. The petition wanted to underline the importance of addressing the needs of rare disease populations to ensure the application of the principles of justice. Health care and treatment for rare diseases is a human rights issue with a global dimension and impact. Specific policies must be put in place to address the needs of people affected by rare diseases and promote universal health coverage across the world as stated by Article 3 of United Nations Sustainable Development Goals: “Ensure healthy lives and promote well-being for all at all ages”. To accomplish this objective, there is also a need for enhanced efforts in the fields of fundamental, translational, epidemiological and clinical research. Public policy plays a crucial role in advancing rare disease research. The Orphan Drug Act of 1983 in the US and the European Regulation n. 141/2000 demonstrate the impact that policy decisions can have in driving forward innovative research and show the successful outcomes that public policy intervention can achieve. However, much more international attention is needed to push forward research and increase prevention, diagnosis and treatments for rare disease patients. National Plans also provide significant emphasis in individual countries.
The petition calls upon public authorities to take the appropriate steps to improve research efforts in the field of Rare Diseases. The need for public support is founded on the accomplishment of the universally recognized right to health, a public good that national authorities must pursue, assuming the role of investors in research when private funders do not.
The following points were provided as guidance for the establishment of policies on rare diseases research:

1. Increase allocation of resources
2. Promote a multi-disciplinary and coordinated approach
3. Adopt specific incentives policies
4. Leverage existing knowledge and optimize the use of existing drugs
5. Widen health economics criteria in considering the cost-effectiveness of RDs research
6. Adopt appropriate and internationally recognized rare disease classification systems
7. Invest in innovative diagnostic methods of rare diseases to enable early intervention
8. Set criteria for ante-natal and newborn screening and ethical controls
9. Recognize the expanding role of patient groups in contributing to develop and foster knowledge and awareness on rare diseases, and include patients at all levels in the development of policies, research agenda and protocols for specific rare diseases

change.org

# 2016 ACTIVITIES
# SUPPORT FOR SCIENTIFIC RESEARCH

## THE RE(ACT)X SWITZERLAND – WE CARE FOR RARE SYMPOSIUM DEVOTED TO SWISS TRANSLATIONAL SCIENCE ON RARE DISEASES

The "RE(ACT)x Switzerland: We Care for Rare" brought together a group of motivated researchers and patients advocates to discuss about the creation of a Swiss Center for Therapeutics Discovery (SCTD) focused on rare diseases (RD). The symposium organized by the BLACKSWAN Foundation on behalf of "Medicine Discovery & Delivery" Health2030 workgroup, will be probably remembered as the turning point for translational research on rare diseases in Switzerland and in Europe.

The event was organized at Campus Biotech in Geneva under the patronage of ProRaris, the Swiss alliance of rare disease patients.

Prof. Patrick Aebischer (EPFL) addressed a welcome message to the more than 100 people representing the Swiss and international research community. The President of EPFL emphasized the importance of building an ecosystem for rare diseases such as the successful one developed by EPFL for the Innovation Park. He also welcomed the commitment of the people who started the SCTD project and offered his full support to make it a reality.

Welcoming the participants, Dr. Olivier Menzel, President and founder of the BLACKSWAN Foundation, illustrated the unmet medical needs for rare diseases and explained how the creation of a Swiss Center for Therapeutics Discovery was a unique opportunity to provide hope for patients and position Switzerland as a pioneer of drug discovery for rare diseases in Europe.

The first part of the day was dedicated to listening to patients' voices reporting on the unmet medical needs, to researchers working in that domain showing the advances that science can bring but also the difficulties experienced in translating scientific knowledge into cure to patients and to learn from successful projects that made it from the bench to the bedside.

Keynote speakers included Mrs. Anne-Françoise Auberson, President of ProRaris, Swiss Rare Disease Alliance; Mrs. Sharon S. Lagas, Board President, Alport Syndrome Foundation, USA; Dr. Olivier Dorchies University of Geneva, Switzerland; Prof. Gisou van der Goot, Dean of the School of Live Science at EPFL and Dr. Rudolf Hausmann, VP Technical Development & Operations at Santhera, Switzerland.

Pr. Kay Davies and Dr. Rashmi Gopal-Srivastava provided institutional examples of rare diseases translational centers and exiting inputs for thoughts to the participants. Professor Davies, Director of the MRC functional genetics unit, governor of the Wellcome Trust and director of the Oxford Centre for Gene Function in the UK brought the model of the Oxford Rare Disease Initiative and suggested to build a taskforce benefiting from clinical and patient networks as well as academic and industrial resources.

Through the experience gained by the National Center for Advancing Translational Science (NCATS) at NIH, in the United States, Dr. Rashmi Gopal-Srivastava Director of Extramural Research Program offered an overview of the Rare Diseases Clinical Research Network (RDCRN) Program.

Thanks to Dr. Michael Foley's presentation on the Tri-Institutional Therapeutics Discovery Institute in the US, the audience had excellent points of discussion for the second part of the RE(ACT)x symposium.

Prof. Leonardo Scapozza, representing the Medicine Discovery and Delivery Health2030 group, presented the project for the creation of a Swiss Center for Therapeutics Discovery and exchanged with the public audience. He received an enthusiastic feedback,

# 2016 ACTIVITIES
# SUPPORT FOR SCIENTIFIC RESEARCH

excellent ideas for the implementation phase and advices to avoid possible bottlenecks.
The event was a major milestone in the creation process of the Swiss Center for Therapeutics Discovery focused on rare diseases. The interest and commitment from the vast majority of the participants demonstrated the viability of the project and confirmed that the main and probably only difficulty lie on the raising of funds for the kickoff.

However, promising signs already came from Swiss academic institutions, the federal government and from private donors bringing hope for the establishment of a public-private partnership in favor of the SCTD. In the coming months, the BLACKSWAN Foundation is going to raise funding for the creation of this unique center which will give hope and treatments to millions of patients.

# 2016 ACTIVITIES
# INITIATIVES TO INCREASE AWARENESS AND COLLECT FUNDS

In July 2016, the Foundation in collaboration with its volunteers (BLACKSWAN Events Geneva) organized a social fundraising event in Geneva on the historical sailing boat “the Neptune”. During this convivial moment, the president of the BLACKSWAN Foundation gave a speech to thank the participants and to increase awareness on rare and orphan diseases.


Le samedi 23 avril le Lions Club de Genève a mis en lumière la Fondation BLACKSWAN lors d’une soirée festive et légère qui s’est tenue dans la salle de congrès de Pelexpo. Plumes, strass et paillettes ont été à l’honneur avec en point d’orgue le spectacle « Cabaret Follies », menée par la très élégante Emma Mylan. Stéphane Lambiel, Franck Bouroullec, Vera Marozava, Satya Oblette ont aussi été de la partie.
Au programme il y a eu un flot de surprise toute au long de la soirée. Un dîner-spectacle, une performance du peintre Franck Bouroullec qui a réalisé une de ses œuvres sur scène (la fabuleuse Marilyn Monroe) et dont la vente aux enchères sur place a permis de faire monter l’adrenaline chez les amateurs d’art. Nous remercions tous les participants, plus de 400, et donateurs qui ont permis de récolter 110’000 francs en faveur de notre Fondation.

# FINANCIAL REPORT 2016

ALBA

Lugano, 24rd August 2017

To the
General meeting of the
**Blackswan Foundation**
**For research on orphan diseases**
Via Cantonale 26
**6948 Porza**

**REPORT OF THE STATUTORY AUDITORS**
**YEAR 2016**

As statutory auditor, we have audited the accounting records and the financial statements (balance sheet, income statement and notes) of **Blackswan Foundation for research on orphan diseases** for the year ending 31 December 2016.

These financial statements are responsibility of the board of directors. Our responsibility is to express an opinion on these financial statements based on our audit. We confirm that we meet the legal requirement concerning professional qualification and independence.

Our audit was conducted in accordance with Swiss Auditing Standards, which require that an audit be planned and performed to obtain reasonable assurance about whether the financial statements are free from material misstatement. We have examined on a test basis evidence supporting the amounts and disclosures in the financial statements. We have also assessed the accounting principles used, significant estimates made and the overall financial statement presentation. We believe that our audit provides a reasonable basis for our opinion.

The Foundation closed the accounting year 2016 with a positiv balance of Chf 130'841.13. The net asset of the Foundation, after capitalizing the annual result turn into a postiv asset balance of the amont Chf. 212'277.24.

In our opinion, the accounting records and financial statements comply with Swiss law and the Foundation's articles.

We recommend that the financial statements submitted to you be approved.

Alba Advisors SA
Dir. Memmi Faya

Enclosures

# FINANCIAL REPORT
# 2016 BALANCE

**BLACKSWAN FONDAZIONE**
Via Cantonale 26
**6948 Porza**

Stampato il 24.08.2017/09:41:46
Contabilità 2016 dal 1.1.2016 al 31.12.2016

## BILANCIO patrimoniale al 31.12.2016

| Conto | Descrizione | Dare (CHF) | Avere (CHF) | (2015 al 31.12.2015) Saldo prec (CHF) | Variazione | |
|---|---|---|---|---|---|---|
| | **ATTIVI** | | | | | |
| **10** | **Sostanza circolante** | | | | | |
| ***100*** | ***Mezzi liquidi*** | | | | | |
| 1020 | Banca BSI c/ 414 AA | 133'152.45 | | 6'517.25 D | 126'635.20 | *999.9* |
| 1021 | Banca BSI c/ 414 AB | 727.55 | | 796.07 D | -68.52 | *-8.6* |
| 1022 | Banca BSI c/ 414 MA | 3'248.15 | | 8.01 D | 3'240.14 | *999.9* |
| 1023 | Banca BSI c/ 414 LA | 12'350.66 | | 9'042.90 D | 3'307.76 | *36.6* |
| 1030 | PayPal | 0.00 | | 240.40 D | -240.40 | *-100.0* |
| 1031 | PayPal EUR | 412.73 | | 294.68 D | 118.05 | *40.1* |
| | ***Totale Mezzi liquidi*** | ***149'891.54*** | ***0.00*** | ***16'899.31*** D | *132'992.23* | *787.0* |
| ***109*** | ***Transitori*** | | | | | |
| 1090 | Transitori attivi | 8'349.65 | | 4'251.35 D | 4'098.30 | *96.4* |
| | ***Totale Transitori*** | ***8'349.65*** | ***0.00*** | ***4'251.35*** D | *4'098.30* | *96.4* |
| | **Totale Sostanza circolante** | **158'241.19** | **0.00** | **21'150.66** D | 137'090.53 | *648.2* |
| **11** | **Sostanza fissa** | | | | | |
| 1105 | Mobilio e macchine ufficio | 3'390.00 | | 4'515.00 D | -1'125.00 | *-24.9* |
| 1106 | Hardware e Software | 5'830.00 | | 2'590.00 D | 3'240.00 | *125.1* |
| 1200 | Attivazione Progetto RARevolution (2016-2019) | 53'250.00 | | 71'000.00 D | -17'750.00 | *-25.0* |
| | **Totale Sostanza fissa** | **62'470.00** | **0.00** | **78'105.00** D | -15'635.00 | *-20.0* |
| | **Totale ATTIVI** | **220'711.19** | **0.00** | **99'255.66** D | 121'455.53 | *122.4* |
| | **PASSIVI** | | | | | |
| **20** | **Capitale di terzi** | | | | | |
| ***200*** | ***Debiti a breve termine*** | | | | | |
| 2000 | Creditori | | 4'634.65 | 8'061.95 A | -3'427.30 | *-42.5* |
| | ***Totale Debiti a breve termine*** | ***0.00*** | ***4'634.65*** | ***8'061.95*** A | *-3'427.30* | *-42.5* |
| ***209*** | ***Transitori*** | | | | | |
| 2090 | Transitori passivi | | 4'390.00 | 4'000.00 A | 390.00 | *9.8* |
| 2093 | Transitorio stipendi | 590.70 | | 242.40 D | 348.30 | *143.7* |
| 2095 | Accantonamento imposte | | 0.00 | 6'000.00 A | -6'000.00 | *-100.0* |
| | ***Totale Transitori*** | ***590.70*** | ***4'390.00*** | ***9'757.60*** A | *-5'958.30* | *-61.1* |
| | **Totale Capitale di terzi** | **590.70** | **9'024.65** | **17'819.55** A | -9'385.60 | *-52.7* |
| **21** | **Capitale proprio** | | | | | |
| 2150 | Risultati riportati | | 81'436.11 | 87'435.47 A | -5'999.36 | *-6.9* |
| 2170 | Risultato d'esercizio | | 130'841.13 | 5'999.36 D | -136'840.49 | *999.9* |
| | **Totale Capitale proprio** | **0.00** | **212'277.24** | **81'436.11** A | 130'841.13 | *160.7* |
| | **Totale PASSIVI** | **590.70** | **221'301.89** | **99'255.66** A | 121'455.53 | *122.4* |
| | ***Totale a pareggio*** | **221'301.89** | **221'301.89** | | | |

# FINANCIAL REPORT
# 2016 INCOME STATEMENT

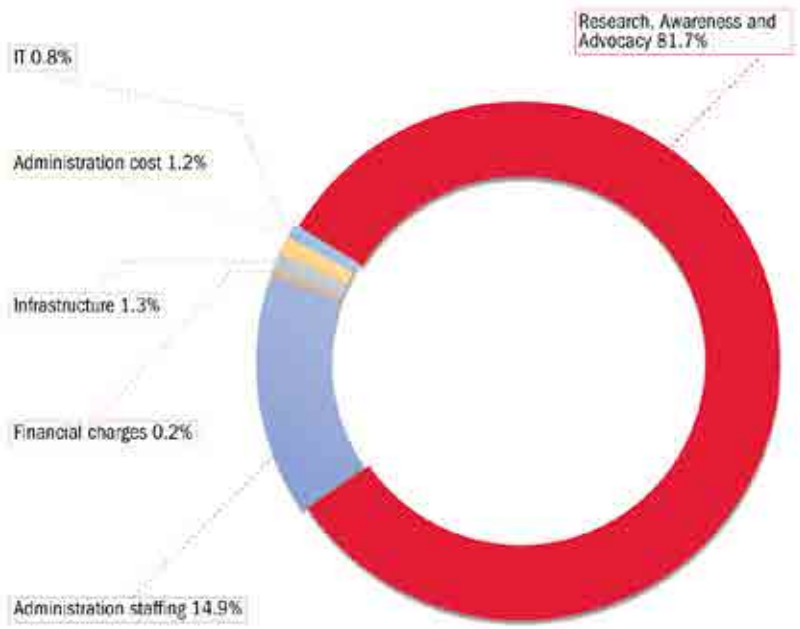
**BLACKSWAN FONDAZIONE**
Via Cantonale 26
**6948 Porza**

Stampato il 24.08.2017/09:42:29
Contabilità 2016 dal 1.1.2016 al 31.12.2016

CONTO economico dal 1.1.2016 al 31.12.2016

| Conto | Descrizione | Dare (CHF) | Avere (CHF) | (2015 dal 1.1.2015 al 31.12.2015) Saldo prec (CHF) | Variazione | |
|---|---|---|---|---|---|---|
| | **COSTI PER MATERIALE E MERCI** | | | | | |
| | **Totale COSTI PER MATERIALE E MERCI** | **0.00** | **0.00** | **0.00** D | 0.00 | *100.0* |
| | **COSTI D'ESERCIZIO** | | | | | |
| **40** | **Costi del personale** | | | | | |
| 4000 | Stipendi e salari | 67'200.00 | | 67'200.00 D | 0.00 | *0.0* |
| 4010 | AVS/AI/IPG | 11'081.65 | | 8'126.00 D | 2'955.65 | *36.4* |
| 4020 | Assicurazione malattia | 1'024.85 | | 786.10 D | 238.75 | *30.4* |
| 4030 | Assicurazione infortuni | 24.55 | | 88.40 D | -63.85 | *-72.2* |
| 4040 | Cassa pensione | 8'022.00 | | 4'840.20 D | 3'181.80 | *65.7* |
| 4050 | Imposte alla fonte | | 255.80 | 25.95 A | 229.85 | *-885.7* |
| | **Totale Costi del personale** | **87'353.05** | **255.80** | **81'014.75** D | 6'082.50 | *7.5* |
| **41** | **ALTRI COSTI D'ESERCIZIO** | | | | | |
| 4210 | Interessi e spese diverse | 214.43 | | 43.87 D | 170.56 | *388.8* |
| 4220 | Spese bancarie | 738.59 | | 660.93 D | 77.66 | *11.8* |
| 4250 | Differenze di cambio | 155.66 | | 95.01 D | 60.65 | *63.8* |
| 4400 | Ammortamenti | 5'010.55 | | 3'232.70 D | 1'777.85 | *55.0* |
| 4401 | Ammortamento progetti | 17'750.00 | | 17'668.89 D | 81.11 | *0.5* |
| 4700 | Materiale ufficio e stampati | 6'644.05 | | 6'000.00 D | 644.05 | *10.7* |
| 4701 | Promozione - stampati | 0.00 | | 318.60 D | -318.60 | *-100.0* |
| 4703 | Spese Gala | 7'800.00 | | 1'300.00 D | 6'500.00 | *500.0* |
| 4704 | Promozione e marketing | 4'719.86 | | 1'944.56 D | 2'775.30 | *142.7* |
| 4705 | Comunicazione visiva (grafica) | 227.00 | | 0.00 D | 227.00 | *100.0* |
| 4720 | Spese telefoniche/fax/postali | 749.05 | | 647.30 D | 101.75 | *15.7* |
| 4730 | Spese TforT | 0.00 | | 0.50 D | -0.50 | *-100.0* |
| 4760 | Spese di rappresentanza | 7'237.74 | | 8'767.17 D | -1'529.43 | *-17.4* |
| 4770 | Costi amministrativi e consulenze | 1'004.22 | | 0.00 D | 1'004.22 | *100.0* |
| 4771 | Tasse, Fiduciaria, membership | 5'799.34 | | 3'832.00 D | 1'967.34 | *51.3* |
| 4802 | Sito web e webmaster | 4'515.10 | | 3'676.16 D | 838.94 | *22.8* |
| 4811 | Contributi alla ricerca | 22'482.30 | | 0.00 D | 22'482.30 | *100.0* |
| 4815 | Spese progetto RAREvolution | 90'000.00 | | 0.00 D | 90'000.00 | *100.0* |
| 4830 | RE(ACT) congress | 121'573.78 | | 66'014.26 D | 55'559.52 | *84.2* |
| 4831 | RE(ACT) Community | 85'350.68 | | 20'342.37 D | 65'008.31 | *319.6* |
| 4832 | RDI (Rare Disease International) | 121'200.00 | | 24'000.00 D | 97'200.00 | *405.0* |
| 4890 | Altri costi d'esercizio | 0.00 | | 100.00 D | -100.00 | *-100.0* |
| 4900 | Imposte | | 6'000.00 | 0.00 D | 6'000.00 | *100.0* |
| | **Totale Altri costi d'esercizio** | **503'172.35** | **6'000.00** | **158'644.32** D | 338'528.03 | *213.4* |
| | **Totale COSTI D'ESERCIZIO** | **590'525.40** | **6'255.80** | **239'659.07** D | 344'610.53 | *143.8* |

# FINANCIAL REPORT
# 2016 EXPENSES OVERVIEW




NOTES TO FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2016

1. ORGANIZATION

The BLACKSWAN Foundation (BSF) is a Swiss Foundation, established as a not-for-profit legal entity registered in Porza under statutes dated 15th April 2010 and in accordance with article 80 and those that follow of the Swiss Civil Code. It is managed by a foundation board. BSF contributes to the development of research on rare and orphan diseases worldwide. The Foundation supports research on all different types of rare and orphan disease, which makes its vision unique and helps in finding new solutions that can assist a large variety of projects. As with all Swiss foundations recognized for international public good, BSF is monitored by the Swiss Federal Supervisory Board for Foundations.

The capital fund is fully subscribed at CHF 50,000 as stipulated under the original legal statutes of BLACKSWAN Foundation.

2. GOVERNANCE

The Foundation Board is the Foundation's supreme body. It takes all decisions necessary or effective for the achievement of the Foundation's aims. It has general responsibility for the affairs of the Foundation and supervises all activities carried out under its authority by the Foundation's other bodies to which power and authority have been delegated.

Members of the Board act on voluntary basis and may not claim any financial compensation other than expenses incurred such as travel and accommodation. For activities over and above the normal requirements of the position, each Member may receive appropriate reimbursement and compensation. Payment of any such reimbursement or compensation is only permissible where it corresponds to a service carried out of for the benefit of the Foundation.

The only employee, Mrs. Chiara Ciriminna Swan (Project Coordinator & External Relations) paid by the Foundation serves on the Board only in an advisory capacity and have no voting rights. Moreover, her salary was covered by unrestricted donations dedicated to support the "administrative costs" of the Foundation.

3. RECOGNITION OF DONATIONS

Contribution from donors (public, private and philanthropic sources) are recognised in the financial statement on an accrual basis when they have been received or confirmed in writing pledges. Contributions which are subject to donor-imposed stipulation for a specific purpose or use in future years may be deferred or attributed to a restricted reserve according to the particular nature of the specific conditions.

# FINANCIAL REPORT
# 2016 INCOME STATEMENT

**BLACKSWAN FONDAZIONE**
Via Cantonale 26
**6948 Porza**

Stampato il 24.08.2017/09:42:29
Contabilità 2016 dal 1.1.2016 al 31.12.2016

## CONTO economico dal 1.1.2016 al 31.12.2016

| Conto | Descrizione | Dare (CHF) | Avere (CHF) | (2015 dal 1.1.2015 al 31.12.2015) Saldo prec (CHF) | Variazione | |
|---|---|---|---|---|---|---|
| | **RICAVI D'ESERCIZIO** | | | | | |
| **60** | **Ricavi da vendite** | | | | | |
| ***600*** | ***Ricavi da vendite*** | | | | | |
| 6001 | Registrazioni RE(ACT) congress | | 80'250.35 | 14'928.58 A | 65'321.77 | *437.6* |
| 6002 | Donazioni RE(ACT) congress | | 10'000.00 | 81'494.76 A | -71'494.76 | *-87.7* |
| 6003 | Donazioni TforT | | 150.00 | 7'219.60 A | -7'069.60 | *-97.9* |
| 6004 | Donazioni libri ricette | | 3'150.00 | 410.00 A | 2'740.00 | *668.3* |
| 6006 | Donazioni PayPal | | 1'482.86 | 1'135.97 A | 346.89 | *30.5* |
| 6008 | Donazioni varie | | 11'033.45 | 31'212.80 A | -20'179.35 | *-64.7* |
| 6009 | Donazioni Libro Rare Disease | | 0.00 | 32.00 A | -32.00 | *-100.0* |
| 6010 | Donazioni progetto RAREvolution | | 485'083.40 | 93'310.00 A | 391'773.40 | *419.9* |
| 6012 | Donazioni Gala/Eventi | | 113'240.00 | 3'900.00 A | 109'340.00 | *999.9* |
| 6013 | Sponsorin RE (ACT) Congress | | 10'720.67 | 0.00 A | 10'720.67 | *100.0* |
| 6050 | Interessi attivi | | 0.00 | 16.00 A | -16.00 | *-100.0* |
| | ***Totale Ricavi da vendite*** | ***0.00*** | ***715'110.73*** | ***233'659.71*** A | *481'451.02* | *206.0* |
| | **Totale Ricavi da vendite** | **0.00** | **715'110.73** | **233'659.71** A | 481'451.02 | *206.0* |
| | **Totale RICAVI D'ESERCIZIO** | **0.00** | **715'110.73** | **233'659.71** A | 481'451.02 | *206.0* |
| | ***Risultato d'esercizio*** | ***130'841.13*** | | ***5'999.36*** A | -136'840.49 | *999.9* |
| | ***Totale a pareggio*** | **721'366.53** | **721'366.53** | | | |

# BLACKSWAN FOUNDATION AFFILIATIONS & PARTNERS

- ProRaris - Swiss rare disease alliance - www.proraris.ch
- EURORDIS - European alliance of patient organizations - www.eurordis.org
- E-RARE - European Consortium of national funding bodies for research on rare diseases - www.erare.eu
- RDI - Rare Disease International - The the global alliance of people living with a rare disease of all nationalities across all rare diseases - www.rarediseasesinternational.org

# MEMBERS OF THE FOUNDATION

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Children's Hospital, Geneva, Switzerland

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Head of the Pediatric Gastroenterology-Hepatology Unit, Catholic University of Leuven, Belgium

## DONATIONS

### ONLINE
www.blackswanfoundation.ch
(secured payment by credit card or PayPal)

### BY BANK TRANSFER
EFG Bank SA
Via Magatti 2
6900 Lugano, Switzerland

IBAN: CH5208667007280511007 (Donation in CHF)
IBAN: CH8308667007280511031 (Donation in Euro)
IBAN: CH3308667007280511058 (Donation in USD)

SWIFT: EFGBCHZZ
SIC: 086673
Clearing: 8667

### DONATION BY SMS (SWITZERLAND ONLY)
### DONATION BY SMS TO 339
BS followed with an amount
(i.e. for an immediate donation of 100 CHF,
send BS 100 to 339).

## CONTACT

BLACKSWAN Foundation
Chemin de la Riaz 11
1418 Vuarrens - Switzerland
Infoline: +41 21 887 64 34
contact@blackswanfoundation.ch

www.blackswanfoundation.ch
www.react-community.org
www.react-congress.org

### FACEBOOK
www.facebook.com/Blackswan.Foundation
www.facebook.com/REACT.community.official

### TWITTER
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twitter.com/react_community

### LINKEDIN
www.linkedin.com/company/blackswan-foundation
www.linkedin.com/grp/home?gid=3830895

### INSTAGRAM
instagram.com/blackswan_foundation/

BLACKSWAN®
FOUNDATION

SWISS FOUNDATION FOR RESEARCH ON ORPHAN DISEASE S
SCHWEIZERISCHE STIFTUNG FÜR DIE FORSCHUNG SE LTENER KRANKHEITEN
FONDATION SUISSE POUR LA RECHERCHE SUR LES MALADIES ORPHELINE S
FONDAZIONE SVIZZERA PER LA RICERCA SULLE MALA TTIE ORFANE