20 Years
European Gaucher Alliance
Driven by passion for patients
A rare commitment

To discover and deliver transformative therapies for patients with rare and special unmet medical needs, providing hope where there was none before.

Celebrating over 20 years of partnership and support with the European Gaucher Alliance.

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Dear Friends

It hardly seems possible that twenty years have passed since that small group of Gaucher patients’ representatives from seven different countries came together in the lobby of the conference suite at the EWGGD meeting in Trieste in 1994. So much has changed for the Gaucher patients, but in so many ways the challenges patients faced then, remain the same. In 1994 discussions were all around two fundamental challenges: to know more about Gaucher disease and to access the available treatment. Twenty years on those aspirations remain at the central core of the activities of the EGA.

In 1994 there was one licensed therapy, one company engaged in the production of treatment, heated discussions on dosage levels and patients in many countries struggling to persuade their local or national health authorities to meet the cost of this revolutionary but expensive therapy. Today there are four licensed therapies (three in Europe) with more in the pipeline and in affluent countries at least, access to therapy is not hampered by its high cost.

In the twenty years that have passed, patients have benefitted from the development of world class centres of excellence headed by brilliant doctors and healthcare professionals and scientific discoveries that have led and will lead to a greater understanding of, and ability to treat Gaucher disease. Together with the doctors and the companies we successfully navigated the global supply shortage and scientists and clinicians are still giving all their waking hours to push back the boundaries of human knowledge to better understand Gaucher disease and in doing so develop new research areas, providing strategies to best benefit patients.

However, twenty years on in many ways the challenges remain the same: to better understand Gaucher disease in order to address the unmet needs, particularly neuronopathic disease and the connections with more common conditions such as Parkinson’s disease and cancer: to ensure that patients who can benefit from treatment wherever they are in the world are able to access the treatment that they need. For the EGA every plea for help must be answered, every child or adult that succumbs to Gaucher disease is a tragedy shared. For as long as these challenges exist the EGA will continue to work tirelessly to meet them.

The letters that follow in this anniversary brochure give praise and thanks, but none of what we have achieved would have been possible without the guidance, support and inspiration we constantly receive from the doctors, scientists, healthcare professionals and industry leaders. Without the initiative of Professor Hans Aerts and Professor Timothy Cox of inviting patients to the first EWGGD the EGA may never have been born and without the engagement of you all we would not have grown to be the voice and the organisation that we have become.

I know I speak for the EGA’s founding members, the current EGA Board and all our country members when I express our deepest gratitude to all members of the “Gaucher family” for everything that you have done over the last twenty years and we look forward to continue working with you in the years ahead for the benefit of Gaucher patients.

With renewed thanks and good wishes
Jeremy Manuel O.B.E.
The European Gaucher Alliance was founded twenty years ago in 1994 during the First Meeting of the European Working Group on Gaucher Disease which was held in Trieste. And it was during this Meeting that a group of seven friends sat around a table, drinking tea and coffee and agreed upon the necessity to have The European Gaucher Alliance. Nothing formal, just a group of good friends who cared for each other and Gaucher patients throughout Europe. We were all so enthusiastic, so excited by what was happening. The names of the people who founded the EGA representing the patient organizations in their countries were: Ghislaine Surrel – Vaincre le Maladies Lysosomales, France; Dorit Levy – Israeli Gaucher Association, Israel; Fernanda Torquati – Associazione Italiana Gaucher, Italy; Ria Guijt – Gaucher Vereniging Nederland, The Netherlands; Ulla Greta Johannson – Morbus Gaucher-foreninge, Sweden and Jeremy Manuel – Gauchers Association, United Kingdom with the help of our beloved Susan Lewis.
We the representatives and signees of the associations mentioned here above, decided to create a working group to represent our mutual interests and goals.

We hereby declare the goals of the Alliance:

- Exchange information with particular emphasis to:
  a) Monitoring and treatment of Gaucher Disease;
  b) Research carried out in member countries and elsewhere;
  c) Keep the members of the patient organizations informed about the development of other member countries;
  d) Encourage international research projects about Gaucher’s disease;
  e) Collaborate actively with the experts members of the European Working Group on Gaucher Disease;
  f) Invite other European countries of patient organisations to join us;
  g) Represent the interests of Gaucher patients within the Common Market, the European Alliance and other specific organizations;
  h) Defend the interests of Gaucher patients no matter what European country they belong to, and in particular:
    i) To guarantee adequate treatment;
    j) To guarantee that the voice of the patient affected with Gaucher disease be listened to wherever is deemed necessary.

I believe we reached many of these goals, although naturally, much still needs to be done. I wish you all my best for your future work and engagement and I herewith wish to thank my friends from the European Gaucher Alliance for 20 years of understanding, support and help.
EGA milestones

1994
• Representatives from patient groups in France, UK, Sweden, Israel, Italy and the Netherlands met at the 1st EWGGD meeting in Trieste and formed the EGA
• Cerezyme receives FDA approval in the USA
• Ceredase receives a license in UK by the Department of Health for use in the treatment of Gaucher patients

1995
• Germany became a member of the EGA

1996
• Chitotriosidase as a new biochemical marker for Gaucher disease is identified by Dr. Hans Aerts of the Amsterdam Medical Centre

1997
• 8 patient groups are represented at the 2nd EWGGD meeting in Maastricht, Netherlands.
• Belgium and Spain joined the EGA

1998
• A book on Gaucher disease edited by Prof. Ari Zimran is published
• Cerezyme is approved by the EMA in February

1999
• 10 patient groups are represented at the 3rd EWGGD meeting in Lemnos, Greece
• The EGA tasks EWGGD to prepare a Consensus paper on the Management of nGD in Lemnos, Greece
• Greece became a member of the EGA

2000
• 11 patient groups are represented at the 4th EWGGD meeting in Jerusalem
• The first EGA meeting was held at the EWGGD in Jerusalem
• Czech Republic, Poland and Ukraine joined the EGA
EGA Board Members are invited to attend focus meetings, scientific meetings, workshops and to sit on patient advisory boards.

2001

- Fernanda Torquati (Italy) and Susan Lewis (UK) attend the Bulgarian patient meeting
- ‘Management of nGD; a European Consensus’ was published in Journal of Inherited Metabolic Diseases in July
- 14 countries attend an EGA meeting in London – ‘Focus on Eastern Europe’
- Romania and Russia became members of the EGA

2002

- The European Commission grants marketing authorisation under exceptional circumstances for Zavesca in November – the first oral treatment for Type 1 Gaucher disease patients
- The 5th EWGGD meeting takes place in Prague, Czech Republic attended by 15 country representatives
- Austria and Denmark become members of the EGA
- Susan Lewis participated in the founding meeting for the Danish Gaucher Association in November

2003

- Zavesca is approved for Type 1 Gaucher disease patients by the FDA in August
- EGA representatives have breakfast with Henri Termeer, CEO of Genzyme to discuss access to treatment for Eastern European patients
- Susan Lewis attends the All Ukraine Gaucher Association patient meeting in Kiev, Ukraine in December.
- Susan Lewis attends the Romanian Foundation for Lysosomal Diseases meeting in July
- Serbia and Slovenia became members of the EGA
- The EGA produce a guide on ‘How to form a patient association’
2004

- A position statement on the role of Zavesca is published in the Journal of Inherited Metabolic Diseases co-authored by EGA and EWGGD representatives
- 15 EGA representatives meet in Holland
- Following a meeting with Henri Termeer, the EGA put forward recommendations regarding support for patients in central Eastern Europe to access ERT
- The 6th EWGGD takes place in Barcelona, Spain and is attended by 17 patient group representatives
- Actelion commences a trial in nGD with Miglustat and ERT
- Genzyme and the EGA work together to develop the European Cerezyme Access Programme (ECAP)
- Genzyme launched the European Cerezyme Access Programme (ECAP) in response to the need identified by the EGA
- Anne-Grethe Lauridsen participated in the annual meeting for the Greek association and gave a presentation on home treatment
- Jordan became a member of the EGA

Slovenia, Serbia and the Czech Republic sail down the Danube in Budapest
Presenting the challenges and unmet needs of patients at meeting all over the world is a key task for the EGA.

Relaxing and networking is always a highlight of the EGA members getting together.

2005
- The Management of nGD, a European Consensus, is reviewed and published in the Journal of Inherited Metabolic Diseases
- The first Russian Patient Association Meeting takes place, attended by 10 representatives from the EGA
- First Nordic Gaucher Meeting for Patients and Families (Denmark, Sweden, Norway, Finland)

2006
- A new book on Gaucher Disease, edited by Profs Ari Zimran and Tony Futerman is published and the EGA distribute free copies to doctors in Eastern Europe
- The 7th EWGGD in Cambridge, UK is attended by 18 patient representatives
- A presentation by the EGA is included for the first time in the EWGGD programme
- Actelion’s Zavesca trial for nGD patients is halted
- A book on Gaucher Disease by Claus Niederau is published
- Norway joins the EGA
- Amicus Therapeutics launch a clinical trial with an oral chaperone for Gaucher disease

2007
- Susan Lewis, founder member of the EGA, dies in May
- Tanya Collin-Histed travels to Bulgaria to attend their patient meeting and clinic with Dr Ashok Vellodi
- The National Gaucher Foundation (NGF) pays tribute to Susan Lewis in October in Atlanta
EGA milestones

2008

- The EGA becomes a formal organisation at the EWGGD in Budapest, Hungary and the first EGA board is elected with Jeremy Manuel as Chairman
- The 8th EWGGD meeting is attended by 24 patient representatives
- The EGA produces the first set of proceedings from the EWGGD meeting
- Tanya Collin-Histed travels to Serbia to attend their patient meeting and clinic with Dr Ashok Vellodi
- Estonia, Finland and Latvia join the EGA as members

2009

- Global shortage of Cerezyme ERT announced
- Jeremy Manuel and Tanya Collin-Histed travel to Bad Honeff, Germany to discuss the allocation of available ERT for Gaucher patients with the pharmaceutical companies and the EWGGD
- The first EGA Board meeting is held in Holland
- Ria Guijt receives a knighthood from the Queen of the Netherlands
- The 1st Gaucher Leadership Forum is held in Milan, Italy
- Cerezyme crisis paper – Force Majeure: therapeutic measures in response to restricted supply of imiglucerase (Cerezyme®) for patients with Gaucher disease is published
- European Emergency Cerezyme access programme is launched
- ‘Guidelines for the restart of imiglucerase in patients with Gaucher Disease: Recommendations from the European Working Group on Gaucher disease’ is published in Blood Cells, Molecules and Diseases journal
- The EGA is formally launched in European Parliament
- South Africa became a member of the EGA
- Anne-Grethe Lauridsen attends the Eurordis Membership Meeting and the 1st Council of European Rare Disease Federations in Athens in May
- Amicus Therapeutics announce that the Phase II studies on their oral chaperone for Gaucher disease will not progress to phase III
The EGA’s involvement to Company Advisory Board meetings is key to the successful relationships we have with Industry.

2010
- VPRIV receives FDA approval in the USA in February
- European approval of Velaglucerase Alfa (VPRV) is granted for treatment in Type I Gaucher patients in August
- The 9th EWGGD meeting takes place in Cologne, Germany and is attended by 40 patient representatives from 28 EGA member countries
- In October the 2nd Gaucher Leadership Forum takes place in London, UK
- Lithuania and USA became members of the EGA
- Dr Peter Liese, MEP, becomes a Patron of the EGA

2011
- In January Tanya Collin-Histed visits Gaucher clinics in Chennai and Mumbai, India
- The EGA and EWGGD convene a meeting in Amsterdam with all the pharmaceutical companies to discuss a global Humanitarian Aid pathway
- The EGA launches its members newsletter
- In June members of the EGA board travelled to Boston, USA to visit Shire’s new plant in Lexington
- In September the 3rd Gaucher Leadership Forum takes place in Budapest, Hungary
- The EGA opens an office in Gloucestershire, UK
- In November the EGA launches its website
- A regional patient meeting was held in Čatež, Slovenia in November and was attended by more than 50 people from six countries: Slovenia, Croatia, Bosnia & Herzegovina, Macedonia, Bulgaria and Italy
- In December the EGA becomes officially eligible to be involved in the activities of the EMA
EGA milestones

2012

- In January the EGA employs an administrative assistant to support its activities
- In February an EGA Board meeting is held in London, UK
- In March, the 2nd Humanitarian Aid pathway meeting takes place in Cambridge, UK
- In May Elelyso (Taliglucerase alfa) received FDA approval
- Anne-Grethe Lauridsen attends 6th European Conference on Rare Diseases & Orphan Products in Brussels, Belgium
- The EGA has its first poster presentation in Brussels at the European Conference on Rare Diseases & Orphan Products
- The EGA are invited to speak at Shire’s “Connect for Care” meeting in Brussels
- In June the 10th EWGGD meeting takes place in Paris and is attended by 40 EGA members representing 30 countries
- EGA develops Code of Practice for members to adopt on relationships with the Pharmaceutical Industry
- In October Protalix receives marketing authorisation for Elelyso for the treatment of Gaucher disease from the Israeli Ministry of Health
- The 3rd Humanitarian Aid pathway meeting takes place in Paris, France
- In September, the 4th Gaucher Leadership Forum takes place in Munich, Germany and the EGA board hold a meeting
- Macedonia, Mexico, Bosnia & Herzegovina, India, Slovakia and Switzerland join the EGA as members
- In November the first Go with Gaucher meeting takes place in Frankfurt, Germany and is attended by 31 young adult patients (Types I and III) from 20 countries in Europe, South Africa, North America and Latin America
- The European Commission denies Taliglucerase (Elelyso) market approval for Type I Gaucher Disease
- The growing membership of the EGA is a tribute to its success, members are identified by their country flags

2012–2014
The EGA Board meets face to face 3 or 4 times a year as well as having regular teleconferences.

The commitment by Board members is reflected in the EGA’s slogan ‘passion for patients’. Board members work tirelessly to implement the annual work programme.

2013

- The EGA develops a database to monitor requests for Humanitarian Aid.
- The 1st Pakistan video clinic takes place with Great Ormond Street Hospital in London.
- Tanya Collin-Histed visits three Gaucher centres in India.
- Shire launch their Indian Compassionate Access Programme.
- In October Tanya Collin-Histed presents at a meeting organised by Genzyme in Dubai to help develop patient groups in the Middle East and North Africa.
- Canada, Croatia, Paraguay, Moldova and Luxembourg join the EGA as members.
- In November the EGA becomes a full member of Eurordis.

2014 so far...

- The EGA produce a poster to present the results from its project to take forward the next generation of leaders nationally and internationally (Go With Gaucher).
- Shire launch their global compassionate access programme.
- In March, the 1st India Gaucher video clinic takes place with GOSH, London.
- In April the Humanitarian Aid expert group meet in London.
- The EGA produces a poster to publicise its paper: a survey of member patient organisation’s activities, healthcare environments and concerns.
- Jeremy Manuel and Anne-Grethe Lauridsen attend the European Conference on Rare Diseases & Orphan Products meeting in Berlin, Germany where they present the two EGA posters.
- In May a small group of adult patients meet in Frankfurt, Germany for the second time to take forward the Go With Gaucher initiative.
- In June, the 11th EWGGD meeting takes place in Haifa, Israel and is attended by 40 EGA members representing 30 countries.
- The EGA holds its bi-annual general meeting in Haifa.
- On 26th July, the EGA launches the 1st International Gaucher Day.
Dear friends,

Helen Michelakakis and myself would like to congratulate the EGA on their 20th anniversary that will be celebrated during our biannual meeting. Truly, it was a challenge this time to gather around Hannah Rosenbaums’ group in the beautiful surroundings of the hill that is overlooking the Haifa bay and the famous Bahá’i gardens.

The 11th Workshop of the EWGGD (European Working Group on Gaucher Disease) is now finishing the 3rd day and we have already experienced the spirit of novelty. Although the backbone of our meeting is still based on the contributions by established groups from basic research and clinics, we have systematically tried to approach and encourage younger scientists and clinicians to present their results during poster and oral sessions, with some success.

As in the former years, the principal aim of the meeting is to enable a fruitful international scientific exchange on Gaucher-related issues. The opportunity for presenting unpublished scientific data as well as free discussion is a central premise of the Group and was taken to present the forefront of basic research and clinical advances in Gaucher disease.

What were the mutual projects in the past? We can be proud of the management of the drug shortage period in the years 2009–2011, where we were able to formulate sound rules for the application of the residual enzyme to those patients who were in most need of it. The EGA has helped us a lot in creating a confident atmosphere in a period that has had challenges for most of our patients.

Further, during three consecutive EWGGD meetings the EGA has provided valuable input and helped scientists and doctors and nurses keep in close touch to the basic purpose of their activities, i.e. to help patients live a better life. Since the official acknowledgement of the EWGGD as a scientific association, one EGA representative is a designed member of the Executive Board of this group and patients are regular presenters during some part of the regular meeting.

One of the most astonishing observations is the relative stability of the growing group of individuals who represent the EGA on a national level. In times where everything is supposed to change at a relatively high rate, the continuity of individuals is a pleasant fact that helps all of us in managing Gaucher-related issues. Please stay on.

The role of the EGA is increasing and the success of the meetings has inspired us to forward this association and help increase knowledge on Gaucher’s disease and thereby improve our patients’ lives. We wish you a good future and the sustaining will to hold through and fulfil your individual destinations.

Yours very sincerely,

Prof. Stephan vom Dahl,
Dr. Helen Michelakakis
Dear members of the European Gaucher Alliance,

We are very proud to host you in the summer of 2014 in Haifa, Israel. We in Israel appreciate your dedication to the Gaucher patients in almost 40 countries. We are happy to celebrate with you the 20th anniversary of the EGA during the 11th EWDDGD held in Haifa on the 26th of June 2014. We wish you many more years of fruitful achievements in the field. Together with your warmth, humanity and efforts I wish for more scientific innovations and research progress in Gaucher disease. Your goal is our command to improve treatments and quality of patient’s life. As we say: Whoever saves one life saves the entire world.

Mazal tov and all the best

Hanna Rosenbaum and the Gaucher clinic staff

Rambam Medical Center Haifa, Israel
Congratulations to us, to our self - we who are the EGA. Thank you to the pioneers, the founding members of the EGA. Thank you to the doctors and scientists who invited patient groups to attend the very first EWGGD and who have played an active role in supporting and encouraging the work of the EGA. We who are the EGA today have an obligation to bring the EGA further – to keep in mind that our fare most important goal is to support Gaucher patients who ever they are and where ever they are. Gaucher Association Denmark is a proud member of the EGA and we will continue to support the work for Gaucher Patients not only in Europe but worldwide.

Anne-Grethe Lauridsen, Chairman Gaucher Foreningen Danmark

The EGA’s work programme is developed after the bi annual EGA members meeting to ensure that it reflects the challenges and issues identified by its members.

At EGA meeting 10 years ago, I firstly met Gaucher patients from other countries, as well as physicians and researchers. It is still very encouraging and enriching for me when we share the experience of living with Gaucher disease. I am grateful that I can be part of such a great community.

Irena Znidar, Chairwoman Slovenia Gaucher Association
The EGA has proven to be a lifesaver for our friends all in the Pan-European region, a guidance for those who are seeking information and a great facilitator and stimulator to bridge the gap between the pharmaceutical world and the local patient-organisations.

Best regards,
Jan Timmerman
The Netherlands

“When I first understood what Gaucher means, I was feeling scared and alone ... becoming a part of EGA made me feel as a part of a very large family all over the world. Now I say that Gaucher is the best thing that happened in my life.”

Vesna Aleksovska
Macedonian Rare Disease Association

EGA members at the 7th EWGGD meeting in Paris. The EGA meet prior to the EWGGD meeting to hold its biannual members meeting

EGA meeting
Paris, June 2012
My adventure with Gauchers disease began more than thirty years ago when as a young physician I started working at the Department of Metabolic Diseases at the Memorial Children Health Institute in Warsaw. The head of the Department announced that I was to deal with lysosomal diseases. I considered the news quite grim as in 1980 nothing was really “going on” in the lysosomal diseases. When I asked her what to do with the patients, she said there was diagnostics – and in fact we did have a lysosomal diseases diagnostic laboratory fantastically run by Dr. Barbara Czartoryska – and that I could support and console the affected.

And so I started diagnosing, consoling and supporting both the patients and their families, even though I was dreaming of treatment, trials and many praiseworthy acts flashing through the mind of a young doctor. I was comforted by the fact that at that time I learnt a lot not only from the scarcely available publications, but also from the patients themselves and their families.

My first patients were type 3 Gauchers disease patients – skinny, enfeebled children, some with enormous livers, usually already without spleens. When struck by an imploring sight of one of the patient’s parents asking “what’s next?”, I replied that if we had the cure, Mariusz definitely would be treated. It was back in 1983 and I had no idea what I was talking about. It really was nothing more but a quite irresponsible consolation. And it lasted till the nineties.

The spleen of Wojtek, another patient of mine, was alarmingly large. Dangerous hypersplenism was increasing. He had a brush with splenectomy. And then we heard that “these patients are treated”. There really was a drug. It was a real substitution drug based on the principle “no enzyme – supplement the enzyme and the effects will come.”

“I am going to raise money,” said Wojtek’s mother with determination characteristic of the mothers of the affected children. She raised money for three months of treatment. And then a representative of Genzyme visited us, a dynamic and smiling doctor Ute Stolzle from Germany. She referred to us as “my territory”, which sounded quite ambiguous to us. I had no idea what a pharmaceutical company representative was as we had had no previous contacts with such persons, especially with regards to metabolic disorders, the area of no therapeutic options but for phenylketonuria.

We had to start with the most affected ones… I chose 6 children of my flock, 4 of whom were type 3 of the disease, with no neurological symptoms developed at that time.

It was time to present the case to the Ministry of Health. I can recall that day very well… it was December 8, 1994. Twenty years ago I had neither courage nor self-confidence. I knew that the drug was great from literature and that the children are very sick. I confronted four officials at the Ministry and described the disease and how the drug works. Let me say it again - I explained everything orally, not in writing (it would be impossible today).

The response was that the drug would be purchased “for those patients of yours”. The money was provided. The purchasing procedure was initiated. We started treatment in June 1995, three months after Wojtek began taking the drug bought with the money raised by his parents.

A couple of months later another boy was diagnosed - a terribly ravaged two-year old with a huge liver and spleen and anemia. Unfortunately, he was not in the “approved” group of children. There was no money for his treatment. What could we do?
I told the mother to go to the Ministry and ask for treatment for the child. She went there holding her little Rafał (at that time one could easily access the office, there was no “gates” to go through). Regrettably, it was too late… “Maybe next year” was the answer. The child was in a very bad condition. The mother started crying. Seeing his mother’s tears, Rafał burst out crying, too. To comfort him, the official gave him a colorful pen. The mother took the pen from the baby’s hands, returned it, and said “he needs no pen, he needs the drug”. Soon after an approval to buy the drug for Rafał was signed. As it happens in type 3 disease patients, after one year of therapy the liver and spleen size reduction was spectacular. I took pictures and showed them around to demonstrate the effectiveness of the drug to other patients.

Ute Stolzle was delighted with our patients and often called asking how they were. One day, she called and said “Anna, how about going to Russia? There too must be patients that should be treated.” I said to myself, “crazy”. I tried to dampen her enthusiasm but still I went with her. At first, it was a bit “stiff”… Few knew about the disease, despite the fact that the Moscow laboratory run by Professor Krasnopolskaya was diagnosing patients with Gauchers disease.

It was hard to convince hematologists that bone marrow biopsy was not the only diagnostic method. I sensed some aversion and reluctance. At subsequent visits to Moscow, we met the patients and I spoke to them in Russian. We bonded quickly. I can remember Alene, a few years old pretty girl who was the first to receive treatment in Petersburg under the supervision of Dr Belogurova. We had several more visits to Moscow, several more years passed, and… a complete success – all patients affected in Russia are administered treatment. They have a thriving Gauchers Disease Patient Society. I also accompanied Ute at trips to other Central and Eastern European countries, even to Georgia, where a girl with type 3 disease was diagnosed.

I established an especially close cooperation with Lithuania. Doctor Grażyna Galewska-Kleinotiene, after being awarded a scholarship, developed a doctoral thesis drawing up a genotype/phenotype characteristics and treatment outcomes in Polish and Lithuanian populations of Gauchers disease patients, which she successfully defended in 2009.

In the meantime, in Poland we were already treating all patients diagnosed with type 1 and type 3 of the disease, both children and adults. The adult patients were under our pediatric care. Physicians from the neighboring and other countries would come to Warsaw to attend training sessions and meetings with the patients.

We have a large number of type 3, quite moderate disease resembling the Norrbottian type patients. Due to very discrete nature of the neurological symptoms observed in the patients, with excellent visceral and hematological outcomes, we may say that the therapeutic success obtained in this group is as big as in patients with type 1.

Nowadays, Gauchers disease no longer is an “unknown or little known” one. It is famous thanks to the therapeutic success. When I recollect the 1980s and the feeling of disappointment I had when I was allotted to lysosomal diseases, I can say that in medicine we sometimes can find some “ugly ducklings” that with time mature into beautiful swans.
When Tanya and Jeremy asked me to write a brief contribution to celebrate the 20th Anniversary of the formation of EGA, I have to admit, I was astonished at first. Dealing with the passing of time is always astonishing.

We met 20 years ago in Trieste, at the first European meeting on Gaucher disease, and from that moment we went through many experiences, many different paths that have intertwined and still intertwine around a common point: to gain better knowledge about the disease and to improve its treatment.

I am now recalling my personal experience with Gaucher disease, starting from the late 80es of the past century (a century ago!), when, before ERT, we thought about transplanting amniotic cells as a reservoir of external enzyme, up to today’s abundance of therapies: 4 available enzyme for ERT and 2 small oral molecules!

I bear in my mind the vivid image of 2nd May 1991, when in Trieste, for the first time, we infused placenta enzyme in 4 children with GD3. Three of them are now adults, one child finished high school, and now she works and is a mother, one child is a University student, another child, having a slight intellectual disability, lives with her parents.

Maria left us many years ago, after fighting with all her strength against the disease and after a long calm period with her loving family. It has been a professional adventure indeed, but mainly an experience of life. I met many Maria with their families and many colleagues with whom we became a scientific family. Together we fought for the patients’ right to the therapies, and I will be keeping in my heart the scientific discoveries we achieved.

The daily hurry in our professional and personal lives prevent us from reflecting on what we lived and reached. If we look back to these years we must be proud of what we made together. This is the right way to look at the future, the right way to break, as human beings of this world, the barriers of our minds and the knowledge that restrict us.

This is the meaning of my deep gratitude to the Gaucher community, for the values and the human experience that you transmit to me, and a memory of a friend, Susan Lewis, we shared the intensity of our actions with.

Dr. Bruno Bembi
Bruno Sepodes

Dear Chairman Manuel

I would like to take this special occasion to congratulate the European Gaucher Alliance (EGA) on the occasion of the 20th Anniversary of the formation of this alliance. This has been such a remarkable accomplishment bringing light to a path not always easy to follow. Thanks to the efforts of EGA on joining different patient associations in more than 40 countries, the interests, the voice, and the hopes of those affected by Gauchers Disease have been heard louder and, although the work endures, it is amazing how much the EGA has redefined reality and actively participated in the way we clinically approach these patients and the disease, but also help to construct the scientific knowledge of lysosomal storage disorders today. On behalf of the Committee of Orphan Medicinal Products and myself, I would sincerely like to congratulate you on this occasion and to join EGA on the celebrations of this milestone. I hope that the challenges of the future continue to turn into successful conquests. You have our admiration, appreciation and support for the projects you have ahead in the future you will successfully continue to conquer.

Roscoe O. Brady

Dear Chairman Manuel

I wish to extend my congratulations to the European Gaucher Alliance on its 20th anniversary and to indicate my appreciation for all you have done to support patients with Gaucher disease throughout the world and facilitating their acquisition of effective therapy. The striking improvement in the quality of their lives and expectation of continued benefit from interacting with EGA has been a major influence on their well being. I am certain that the EGA will continue to be a strong contributor to the welfare of Gaucher patients for many years in the future.

I thank you and the members of EGA for your devotion to this momentous endeavor.

Sincerely, 

Roscoe Brady
Dear Jeremy,

I would like to convey my best wishes and my sincere congratulations to the European Gaucher Alliance on their 20th Anniversary!

The Gaucher patient organizations have been an enormous stimulus for the development of treatment and collaborations in the EU and beyond over more than 25 years. The launch of the Alliance in 1994 has further strengthened the position of these organisations. This has resulted in impressive achievements, such as the development of humanitarian aid programs. EGA members continue to play a pivotal role in pushing these programs forward for the benefit of patients in less fortunate areas of the world. The EGA website and meetings provide very valuable information to patients, families and healthcare professionals about the disease.

Through relationships with EU-institutions, the EGA has gained an important position in discussions on orphan drugs and regulations facilitating access to treatment. During the shortage of Cerezyme, the EGA’s role has been crucial to support the fair distribution of remaining enzyme to those patients at highest risk of developing complications. I have been very impressed with the dedication and the professionalism of the EGA. It has been my privilege to be able to work with you and I truly hope and expect that we will continue our collaborations in the future!

Rhonda P. Buyers

Dear Jeremy

Congratulations on 20 years of amazing work not only in Europe, but all over the world! The EGA has had a tremendous positive impact on the Gaucher community. Its leadership and members represent some of the most compassionate, driven individuals in the world and we are proud to be part of such a wonderful organization.

Sincerely,

Rhonda P. Buyers
Dear Tanya,

It is my pleasure to recognize the 20th anniversary of the European Gaucher Alliance. How the world has changed and what a critical role the Alliance has played in transforming the lives of individuals and families affected by Gaucher Disease. With the availability of Ceredase in 1991, the hopes and expectations of the Gaucher community changed dramatically. Our understanding of the disease and how it impacted individual patients and their families rapidly accelerated. The founding European leaders of the alliance responded to the needs of their local community and then quite naturally pioneered a regional model – championing the needs of those less fortunate, learning from each other and providing hope and support to all who reached out.

At Genzyme, we have learned from the EGA, appreciated their thoughtful guidance, taken inspiration from their fierce commitment to the cause, and most importantly, valued the high level of trust and mutual respect. It has been a privilege to be a partner on this 20 plus year journey to improve the lives of all people affected by Gaucher Disease. As we look toward our future, we are a better organization because our journey is shared with you. Together, our work continues.

Sincerely,

Susan Lewis (UK), Ria Guijt (Netherlands) and Fernanda Torquati (Italy)
George Sinca

Romanian Foundation for Lysosomal Diseases has existed for 16 years but the results obtained in these years are very good. It should be noted that it was possible with special support from the EGA. It all started with the integration of our foundation in EGA and when leading EGA members attended the patients day from Romania 4 – 5 - July -2003, we felt that we are not alone. Susan Lewis and the other representatives of the countries of Western Europe animate us in our fight with the rusty and old and we have shown that it can. During this period I had with a team of doctors headed by Ms. Professor Dr. Paula Grigorescu Sido, who with great professional outfit and devotion to patients with Gaucher disease were our support medical science. It was not easy and still is not easy, but we have the satisfaction that all patients diagnosed with Gaucher disease receiving treatment and their quality of life has improved. We hope that the world still all patients will benefit from treatment and that EGA will still be with us and for us. At the core of our business are the words Nothing for us without us, healthy and sick partners for life,"
Flemming Ornskov

On behalf of everyone at Shire, I want to congratulate the European Gaucher Alliance on 20 years of helping improve the lives of Gaucher patients. The passion and commitment of everyone at the EGA has made an immeasurable difference to the lives of patients and families. We are honored to have collaborated with the EGA over many years and commend your efforts to improve understanding, management and treatment of Gaucher disease over the last two decades.

We believe in the importance of partnerships in order to improve the understanding of rare genetic diseases, with the ultimate aim of developing new and effective treatments for orphan diseases. Together with the EGA, we will continue to ensure that Gaucher patients and their families have access to our innovative therapy and the support they need to lead better lives.

We share the EGA’s commitment to the Gaucher community, and look forward to working together in the years to come to help more people in more places lead better lives.

Lorraine Mitchell (Australia), Johanna Parkinnen (Finland), Anne-Grethe Lauridsen (Denmark) and Kate Theoharris (Greece) at the Genzyme Global Patient Group Summit in Framingham and Cambridge, MA (Oct. 2011)
Dear friends,

In 2000 Russian Gaucher Association became a member of the European Gaucher Alliance (ECA). It was a great event for us as we started to closely interact with all European organizations of patients with Gaucher disease. ECA support and collaboration helped us to better understand how to act in our country to make the quality of lives of patients with Gaucher disease and their families in Russia better.

On behalf of all Russian patients with Gaucher disease, please accept our gratitude for the support and cooperation!

From Russia, with love.

Marina Terekhova
Russian Gaucher Association
President
Dear Friends,

Thank you very much for inviting me to participate in the celebration of the 20th Anniversary of the formation of the European Gaucher Alliance. It is an honour and a privilege.

Over the years as a scientist, involved in the study of Gaucher disease as well as through my participation in EWGGD, I have followed with admiration and respect the efforts and steps taken by the European Patient Groups to ensure a better life for patients with Gaucher Disease. The European Gaucher Alliance united all these efforts, strengthening the voice of patients. With 41 member countries EGA is not only a pan-European umbrella group but a global one: “driven by passion for patients”

The achievements of EGA in the last twenty years have been enormous, changing the scene for patients, all over the world. To mention only two: EGA has successfully engaged scientists and pharma in the development of a global structure for the provision of humanitarian aid to patients who are unable to access treatment and is paving the way for the future generations engaging in activities focused on young adult Gaucher Patients.

As a founding member and vice Chairman of EWG GD I am grateful to EGA for keeping me close to the needs of patients and for joining forces to achieve the best solutions possible.

I am sure that in these demanding times with your determination and commitment you will be successful in dealing with the challenges that still lie ahead.

Thank you for all your efforts and inspiration.

With Best Wishes

Helen Michelakakis PhD
It is a great pleasure to congratulate you on your 20th Anniversary! This longevity is an important recognition of the value of the organization for the global Gaucher community.

I became introduced to the EGA in the very early days, which were also the very early days of Genzyme and the availability of the Ceredase/Cerezyme therapy. At that time there was no real infrastructure around this difficult ultra-rare chronic disease. The emergence of the EGA responded to that situation and interestingly, still today has the same critical role. The very nature of ultra-rare diseases is that they don’t have a critical mass in society. Without very strong patient advocacy the needs of these communities would get lost.

Over the past 20 years, the EGA has reached out to scientists, academic medical centers, physicians, governments, insurance companies, providers and industry. The organization has explained and is continuing to explain the need of “shared” responsibility for all the stakeholders. For everyone the goal is to assure sustained access to treatment for patients. To manage the burden of disease in the best possible way, the EGA has taken on this task in a very serious and continuous manner.

I personally remember occasions meeting with the EGA leadership and patient representatives of Eastern European countries. I specifically recall a meeting in Lage Vuursche, in The Netherlands, at the occasion of the 20th anniversary of the Dutch Gaucher Society. It was a spirited meeting, emotional and practical. Out of it became a determination for action. For Genzyme it meant the creation of the European Cerezyme Access Program (ECAP), a humanitarian program which under the guidance of a Medical Advisory Board would create access to the therapy in a very holistic manner. It is but one example of the many very constructive and compassionate initiatives that have come out of the activities of this unusual organization.

“Shared” responsibility is still central to patient advocacy and this is becoming increasingly evident with the availability of therapies for other rare diseases around the world. It is, of course, magnificent that the revolution in medical sciences is starting to offer many new therapeutic options. And in an increasing number of situations the challenge is moving to access. To enable access, patient organizations have a very important role. They are in the best position to set priorities and develop creative solutions. The example delivered by the EGA over the past 20 years is a great role model for the many new ultra-rare patient advocacy organizations that are emerging. The world is grateful for this great contribution.

Wishing the EGA many many more years.

Kindest regards, with admiration

Henri Termeer
It is a real pleasure to send my heartfelt congratulations to The European Gaucher Alliance as it celebrates 20 years of vital and inspiring support of all those associated with Gaucher Disease across Europe.

Susan and David Lewis were regular delegates at our RTMDC (now CLIMB) conferences more than 20 years ago, while I well remember attending the foundation meeting of the fledgling Gaucher Association where I also had the pleasure of meeting Jeremy Manuel for the first time.

With patients scattered across the world, there was swift recognition of the need to develop an Alliance that incorporated not only Gaucher support groups throughout Europe but also the need for the best academic and clinical brains to collaborate their research efforts and clinical expertise and an industry that would foster the development of life-changing orphan medicinal products. This multi-stakeholder approach has enabled EGA to meet the challenges of supply shortages and other events that could otherwise impact on patient health. The programme of humanitarian aid for the most severe patients in the more remote countries is also to be applauded.

EGA has secured a strong and united voice for those living with Gaucher Disease and given them new hope for more effective treatments and an improved quality of life for the future. Consequently, it is a real delight for me to give voice to my admiration for EGA’s far-reaching achievements during the last 20 years and record my warmest wishes for its continued success for many decades to come.
May 14, 2014

Dear Friends at the European Gaucher Alliance,

We at the Gaucher Clinic at Shaare Zedek Medical Center in Jerusalem would like to salute you in celebrating 20 years of achievements as a premier example of an active Patients’ Association. We have seen your group grow from a dedicated few with a vision of pan-European collaboration to a large group of serious and compassionate individuals who are a mainstay of the European Gaucher Alliance. Indeed, We have watched you encompass support and outreach programs not the least of which is an active voice in the proceedings of the EWGGD and in dialogues with various pharmaceutical companies to introduce and sustain compassionate use programs. But above all, we acknowledge the many milestones in patient care that were initiated by the European Gaucher Association, the hallmark of each being sensitivity to the evolving nature of patients’ conditions and aspirations. We wish you and your very capable Chairman, Mr. Jeremy Manuel, along with your thoughtful directors, much success in the future as the community of patients with Gaucher disease grapples with the new challenges of treatment options and improving quality of life.

Most sincerely and with warmest wishes,

Deborah Elstein and Ari Zimran
Dear EGA members,

It is a great and special honor for me to congratulate the EGA on this special occasion of its 20th anniversary.

I still remember the first EWGGD meeting in Maastricht, where a few representatives from different national Gaucher Foundations gathered (and enjoyed the sun on the lawn) and established the European Gaucher Alliance, nowadays an organization representing the interests of Gaucher patient associations from 40 different countries.

You take very serious care of Gaucher patients, not only in the countries from where your representatives come, but also in countries where treatment is not available. You make sure Gaucher patients receive the best treatment, you are very much involved in legislation concerning treatment of Gaucher patients and you are very interested in the scientific and medical advancements in the field of Gaucher disease. I applaud your dedication and your accomplishments.

Since the Maastricht meeting, there hasn’t been an EWGGD meeting without your representatives sharing with us very moving stories that tremendously contribute to the success of the meetings.

I have no words to describe the appreciation the entire community has to your commitment and involvement in Gaucher disease and the EWGGD.

I wish you all many more years of contribution to the Gaucher community and collaboration with the EWGGD.

From the bottom of my heart,

Yours,

Mia Horowitz
Dear Friends,

We are pleased to celebrate the 20th anniversary of the European Gaucher Alliance! A model for the rare disease community, EURORDIS treasures its longstanding engagement with the EGA, standing together to improve conditions for people living with a rare disease. The EGA played a pivotal role in helping patients access the treatments they need via the European Cerezyme Access programme, which has enabled patients to access life-saving treatments. Similarly, the EGA supports patients by providing crucial information — understanding that information equals “empowerment” for people with rare diseases and that patient advocacy is key to improving the rare disease situation. With members in over 40 countries, the EGA has paved the way for people outside of Europe to structure their own patient support groups. A valued member of EURORDIS since 2009, we look forward to continuing our mutual goals on behalf of people with rare diseases everywhere.

Yann Lecam

Jayne C. Gershkowitz

Dear Jeremy,

On behalf of everyone at Amicus Therapeutics, I extend our sincere congratulations to the European Gaucher Alliance on the occasion of your 20th anniversary – a true milestone event. The EGA Board of Directors and its growing membership of country organization leaders are to be applauded for the years dedicated to increasing awareness and understanding of Gaucher disease and serving the international Gaucher community. EGA’s work has made significant contributions to all stakeholders – patients, medical and scientific professionals and industry.

It has been and continues to be a pleasure working with the EGA and strengthening our relationship during the past several years. Amicus appreciates the opportunities for our interactions and to gain your insights.

This is quite an exciting time for those living with Gaucher and other lysosomal storage disorders, and yet many challenges remain. We at Amicus are confident the EGA will head into the future with the same grace, determination and direction that has been your hallmark; you will conquer challenges, turning them into successes. For this journey and the ongoing fulfillment of your important mission, we wish you all the best.

Warm regards,
Dear Jeremy

It is a great pleasure to congratulate the EGA on its 20th Anniversary – and it is astonishing to see how much has been achieved in these two decades.

First, that the EGA and its affiliates now represent a large slice of the developed world. You give a special voice to patients who are, after all, the focus of all activity in the field. Who would have believed that there would now be several enzyme preparations, one approved oral therapy and other treatments in the late stage of development?

I well remember the first meeting in Trieste when there were vigorous discussions with the one supplying company – Genzyme – as the community was seeking transition from a tissue-derived preparation (Ceredase) to an engineered recombinant protein – Cerezyme.

Throughout this period the EGA and its constituent members have not only had a voice, they have taken centre stage, often subtly influencing activities in what has become a substantial international field. During this time, Gaucher disease has taken on a special significance in the ultra-orphan area and has provided a focus for continued exploration by ambitious and inventive Biotech.

More than this, the EGA has not forgotten the intensely personal and human side of its work – seeking at all times to address the larger family of patients in need and in parts of the world where desperately ill patients cannot get active therapy.

Working hand-in-hand with the Genzyme Company first of all, and now in communication with other companies in the field, the EGA, like the good doctor, primarily concentrates on the individual patient needing help.

Congratulations to the ‘organized anarchy’ of the EGA: an independent voice for those who suffer from Gaucher disease - you have not only taken on much work, but have much so much valuable work to do!

All good wishes for the next 20 years!
Dear Jeremy,

Dear friends,

Ladies and Gentlemen,

First of all, I would like to add my best wishes to the voices congratulating the EGA on its anniversary. 20 years – a good age. It is still a great pleasure for me to be able to act as patron of the Alliance. When I was elected as a Member of the European Parliament in 1994 for the first time you laid the foundation for the Alliance in Trieste, Italy. Today we can say the EGA is a successful story. It is necessary and important to have on European level such well-functioning independent representation of interests for the Patients all across Europe, in particular concerning the important role of Europe, the European Union and its legislation for the Patients. I am really proud and happy that we had over the last years significant success in many areas. Milestones of our work were for example the EU “Patients’ rights directive” in 2010. It guarantees that every patient in Europa can get a treatment within all other 28 member states and will be reimbursement to the same conditions as in its own member country. For the treatment of rare diseases we could achieve special conditions. Now, for the patients’ benefit we have to keep the ball running. In my opinion the constant sharpening of awareness and the fight against rare diseases like Morbus Gaucher is a task of utmost importance; I am taking care of on European level.

Due to the small number of patients suffering in the different European countries often it is difficult to get access to information, research, therapies and medicine. Although health policy is more a national duty I am convinced that because of the reasons just mentioned the support and the better awareness for these patients both in public and in politics is merely a European task. One member states cannot cope with challenges occurring from rare diseases.

The success we have had in the past motivates me to stay committed, with forces united also over national borders. I will personally engage that rare diseases like Morbus Gaucher stay in the focus of European politics and hope that our good and fruitful cooperation will lead to improvements for all patients also in the next years. Having all the success we reached in the past years in mind I am sure that joining forces we can reach a lot for the patients suffering from Morbus Gaucher.

Again, congratulations and all the best!

Yours sincerely,

Peter Liese
David Aviezer

Dear EGA leadership and members,

We feel privileged to be able to congratulate the EGA for your 20th anniversary.

Over the many years of working together for the benefit of people with Gaucher and their families, we were exposed to your dedication, enthusiasm but yet rational and thoughtful way of thinking and taking action. Being able to combine forces within Europe for the sake of the European Gaucher community, while keeping in mind those patients around the world who are challenged with obtaining adequate treatment, is a genuine sign of a real community. We have come a long way together, yet there is still a lot to accomplish.

The Protalix team pledges to continue our efforts to provide and develop new and better therapies for the benefit of people with Gaucher, and look forward to an ongoing and long-lasting collaboration with the EGA moving forward to the next era.

Sincerely,
David Aviezer, Ph.D., MBA
President and Chief Executive Officer
Protalix Biotherapeutics, Inc.

Pram Mistry

Dear Jeremy,

20th ANNIVERSARY OF EGA

I am very pleased to send my warmest wishes on the 20th Anniversary of the European Gaucher Alliance. I had the privilege to be at the EWGGD meeting in Trieste in 1994 and meet the pioneering members that went on to develop the most effective advocacy group for Gaucher disease. I have greatly admired the work of EGA across the globe beyond Europe and their fearless advocacy for families affected by Gaucher disease.

I wish you all happy celebrations and will watch with much anticipation the future work of the Alliance.

With best wishes,

Matthias Hess

Dear founders and members of the EGA,

Congratulations to your 20th anniversary! Congratulation to all your efforts during the years since the foundation, which indisputably had an impact on the awareness and today’s understanding of Gauchers Disease and the related challenges and unmet needs – with all this you were and are able to make a real difference for all the families concerned.

We wish you all the best and a lot of success for the next decade, which will hopefully bring new scientific and medical insights as well as improved solutions and hope for all the patients with Gauchers Disease and their relatives.

Best wishes,
Actelion Pharmaceuticals
In memory of and with gratitude to Susan Lewis, July 14 1945 – May 8 2007 – Founding Member of the European Gaucher Alliance who strove tirelessly to make the world a better place for Gaucher Patients
IMPRESSIONS
20 Years EGA
IMPRESSIONS
20 Years EGA
Thank you

On behalf of Amber and all her fellow Gaucher patients, our thanks to the European Gaucher Alliance for their 20 years of advocacy and leadership. At Shire we believe that partnerships are fundamental to the support and care of patients and their families affected by rare diseases. We are honored to work with you and support your mission.

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