The International Thalassemia day was observed at Sri Sathya Sai International Centre on May 8th. This year was special as it was ‘By the Thalassemics and For the Thalassemics’.

The Chief Guest was Mrs. Maneka Sanjay Gandhi, Union Minister for Women and Child Development. She assured all possible support to the society and Thalassemics.

The programme started with lamp lighting and Vandana sung by our talented Swati Tuteja. Our group of children, trained by Shiamak Davar’s institute of Dance performed a Bollywood medley. Then Jyoti Arora (Delhi) and Chandan Das (Orissa), our 2 brilliant authors spoke about their books- Lemon Girl & The Road Taken which have been published and are doing well. Thereafter PriyaNandini from Bangalore gave a fabulous performance along with her father, Lekhraj accompanying her on the guitar. At such a young age PriyaNandini had been singing on stage and receiving many accolades. The evening ended with a rocking performance by Shivangi Amrit from Mumbai. Shivangi has received training at the A R Rahman conservatory and today is an independent musician and an amazing singer.

The Programme was compered by Nehal Dhingra and was attended by more than 500 people including doctors, senior sisters, social workers, a number of people from blood banks and thalassemia families.

(continued on page ............... 4)
“She doesn’t want heart complications in the future.”

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Dear friends,

On behalf of Thalassemics India, it is my privilege to address you through this newsletter.

From February 2015 onwards, we undertook activities with a focus on the spreading of awareness with the help of AOGD and Delhi hospitals. Thalassemics India continues to support the treatment of underprivileged thalassemics individually and also collectively at the Thalassemia unit of St. Stephen’s hospital. Our fund raising event of 18th April, 2015 was a step forward towards our objective of helping the maximum number of thalassemics.

With regard to facilitating communication with our members, we are sharing information about the newer developments and also about our activities through emails, letters, website, face book and e-newsletter.

We keep receiving news from across the country from thalasemia NGOs about their work. We would not only like to thank them for sharing news with us but also congratulate them for the work that they are doing in their respective areas. The major challenge, despite improvements, is still to bridge the gap between State Govt. health bodies, media and Thalassemia NGOs to improve health services in the country for thalassemics and to increase thalassemia awareness in the country.

We do realize that we have to make sure that hopes and expectations of our members are met, despite the ongoing challenges and financial constraints. We are continuously working hard in this direction.

Hope you will find this newsletter interesting. We have added some articles which will give you an insight about the medical advances and the newer therapies.

On behalf of the society, I would like to extend my sincere thanks to all our partners and supporters for their contributions and I look forward to more close and cooperative partnerships in future as well.

Shobha Tuli
Secretary, Thalassemics India
Thalassemics India thanked the performers and the senior sisters by honoring them with gifts. The function was followed by dinner.

The programme was very well covered by the press.

As a part of our efforts on spreading awareness about thalassemia, we had used the medium of the radio to share messages about thalassemia over May 6th, 7th and 8th 2015.

We would like to thank everyone who contributed to the 8th May function, especially Sumit Gupta, Harbans Nagpal, Ashvini Malik, Rekha Arora & Deepak Dhingra.
Over the last few years, there have been dramatic improvements in the management of thalassemia. In the following section, I have attempted to summarize the most recent developments and recommendations in this field sourced from publications in medical literature over the past twelve months. I have divided this into four sections: Chelation, Monitoring, transplantation and gene therapy.

**Chelation**

The focus continues to be on the three chelators available to us: Deferoxamine (Desferal), Deferasirox and Deferiprone (Kelfer). Till the availability of deferasirox, the combination of desferal and kelfer was considered the optimal chelation regimen. With the availability of deferasirox, this has now become the chelator of choice for the majority of patients. However, a proportion of patients do not respond adequately to this drug alone and it was unclear whether other agents could be combined safely with this drug to improve efficacy. There is now emerging data from small studies that, where adequate control of iron overload is not achieved with deferasirox, it can be combined with one of the other two agents. However, it needs to be emphasised that this should be done under close supervision of the treating haematologist and requires meticulous monitoring to avoid any significant side effects.

Another recent study by Dr Gumruk’s group looked at twice daily dosing of deferasirox rather than the usually recommended once daily. This group enrolled patients with thalassemia major who were on the maximal dose of deferasirox at 40 mg/kg/d for more than 6 months and either had S.Ferritin > 1500 or moderate to severe iron loading in cardiac or liver tissues on T2* MRI. They were followed up for a median time of 7 months. At the end of this period, there was more significant reduction in S.Ferritin and improvement in cardiac iron overload. There was no increase in side effects. However, this was a small study and the same would need to be done in larger groups before coming to firm conclusions.

A proportion of patients with thalassemia is Hepatitis C positive and require treatment for this with ribavirin and Peg-Interferon. Until recently, it was unclear whether deferasirox could be safely given in these patients at the same time. Dr Pileri’s group has studied this and their data suggests that deferasirox can be safely continued with ribavirin and Peg-interferon in these patients.

**Monitoring**

We are all well aware that T2* MRI is the modality of choice to assess the iron overload in liver and heart iron overload. However, dedicated software for this is available in very few centres in India. However, Dr Juliano Fernandez has designed an innovative technique, and with the help of this tool iron overload can be evaluated using any MRI machine and the iron overload values can be calculated using a special excel spreadsheet designed by him. There is no cost to this but each centre needs training and some level of experience using this tool. Some centres have started using this tool.

In addition, a recent study by Dr Porter’s team has looked at number of variables in a “Multivariate analysis evaluating potential predictors of cardiac T2* where Cardiac MRI availability is limited”. We know that Serum Ferritin alone does not correlate very well with Cardiac MRI. In this study, it was observed that change in S.Ferritin is the best correlate for improvement in cardiac MRI. Once again, this reemphasises for us that though single values of S.Ferritin may not have a perfect correlation with tissue iron overload but trends in S.Ferritin can give us a very useful picture.

**Bone Marrow Transplant**

Thirty years have elapsed since the first HSCT was performed for patients with Thalassemia Major (TM), and allogeneic transplantation in TM is now accepted as standard clinical practice. In the 1980s and early 1990s, more than 1000 TM patients were transplanted at the transplant center in Pesaro, Italy. They reported a 20-year probability of thalassemia-free survival of 73% in 900 consecutive unselected patients transplanted from an HLA-identical sibling donor. Recent results show that, with modern transplantation approaches, and careful patient selection, even better results could be obtained. At the same time, however, survival without transplantation of TM patients has improved as the result both of a better understanding of the pathophysiology of iron overload and improvements in the medical therapy of TM; survival into the fourth or fifth decade of life is now possible for well-treated patients.

Transplant from a matched sibling donor is considered the best source of BMT. Over the last few years, the use of alternative donor sources such as related or unrelated cord blood, matched unrelated donors and half matched or haploidential donors (usually a parent) has also been actively evaluated. It is very difficult often for families to take a decision. The European Bone Marrow Transplant (EBMT) Inborn
error and EBMT Pediatric Working Parties have brought out a very important document published very recently “Hematopoietic stem cell transplantation in thalassemia major and sickle cell disease: indications and management recommendations from an international expert panel”. This consensus document from the European Blood and Marrow Transplantation Inborn Error Working Party and the Paediatric Diseases Working Party aims to report new data and provide consensus-based recommendations on indications for hematopoietic stem cell transplantation and transplant management.

The committee panel was made up of experts jointly selected by EBMT Inborn Error Working Party and the Paediatric Diseases Working Party. It was composed of 20 members, with recognized clinical and scientific experience in HSCT and/or medical management of TM and SCD. Members came from 18 institutions in 8 countries. Their recommendations are as following:

Young TM patients with an available HLA identical sibling should be

• Offered HSCT as soon as possible before development of iron overload and iron-related tissue damage.
• HLA genoidentical Cord Blood and BM are equally effective stem cell sources.
• Peripheral Blood Stem Cell Transplant should be avoided because of the increased risk of chronic Graft VsHost Disease.
• Assessment of clinical condition according to the Pesaro risk score and adequate transfusions/ chelation regimen are the major issues to be evaluated before deciding to perform HSCT.

Re Haploidentical transplant
• HSCT from an HLA-mismatched family member in TM should still be considered an experimental approach and has to be conducted in the context of well-designed controlled trials only.
• HLA typing of the entire family is advisable. HSCT from an HLA-phenotypically-identical donor is an option to be performed in expert centers.

Unrelated cord blood transplantation

The use of unrelated cord blood transplantation (UCB) as source of stem cells in TM has not been explored in well-designed clinical trials. Evaluation of reported results is hampered by differences in single center experience, conditioning regimens and accepted degree of HLA disparity. Recently, Jaing et al. reported results of UCBT in 35 TM patients. OS was found to be 88%, while DFS was 74%. The cumulative incidence of TRM was 11%. These remarkably good results are likely attributable to the high number of total nucleated cells/kg infused (7.8x10^7/kg). Combining data from 3 different registries, Ruggeri etal. found the outcome of UCBT in TM to be much less favorable; in 35 TM patients, an OS of 69% with a DFS of only 21% was reported. The cumulative incidence of graft failure was 52.4%

The authors concluded that Unrelated cord blood transplantation must be performed only in the context of well-controlled clinical trials in centers with specific UCBT programs.

Gene Therapy

Gene therapy for β-thalassemia has recently seen steadily accelerating progress and has reached crossroads in its development. Presently, data from past and ongoing clinical trials guide the design of further clinical and preclinical studies based on gene augmentation, while fundamental insights into globin switching and new technology developments have inspired the investigation of novel gene-therapy approaches. Moreover, human erythropoietic stem cells from -thalassemia patients have been the cellular targets of choice to date whereas future gene-therapy studies might increasingly draw on induced pluripotent stem cells.

To date, there are a total of seven patients who have been treated successfully or for whom longer follow-up is pending in three clinical trials for β-thalassemia, all of which have used β-globin-expressing lentiviral vectors. The first successful gene therapy trial for β-thalassemia was reported by Cavazzana-Calvo et al in 2010.

Three patients with severe β/β-thalassemia have been treated to date. In the first patient, engraftment of treated bone marrow failed after full myeloablation, requiring reinfusion of backup bone marrow. For the second patient, however, transfection independence was achieved at 12 months after treatment and continues to date.

Engraftment failure for the first patient, a low VCN for the third patient, and oligoclonal reconstitution, vector recombination, and low vector-derived gene expression for the second patient provide important pointers for necessary improvements in future trials.

In summary, gene therapy is one of the most promising approaches for the future treatment of β-thalassemia patients and comprises several, at times complementary, strategies. The clinically most advanced approach, that of substituting nonfunctional endogenous β-globin genes with a normal β-globin gene carried by lentiviral vectors, leads to de novo production of HbA. This approach can be enhanced, as in vitro evidence indicates, by additional treatment with inducers of endogenous Hbf, which is firmly established as clinically beneficial. In the same vein, numerous gene-therapy approaches also draw on Hbf as a positive disease modifier, either by expressing exogenous Hbf from a As a result of
all these developments, and after decades in the making, gene therapy of β-thalassemia has reached a critical phase and is beginning to live up to its long-held promise. At this privileged moment in time, the model systems and protocols are in place to test gene-therapy approaches, and the first clinical trials show therapeutic efficiency and guide our decisions for future developments, such as the choice of conditioning regimen (full or mild), the stem cell source (bone-marrow-derived or mobilized), and the inclusion of insulators for gene augmentation. Ongoing optimization of extant gene-augmentation tools and combinatorial approaches with chemical reagents are approaching therapeutic efficiency, even for severe forms of the disease. At the same time, fundamental insights into globin switching and new tools for cellular reprogramming, transcriptional regulation, post-transcriptional silencing, and genome editing have opened up as-yet uncharted territory in what has become a fast-moving and highly competitive field of research. While there is no telling which approach will win out for widespread clinical application in the course of time, vigilance, widespread competence in shared methodology, and the availability of diametrically different treatment strategies will provide the pressure and scope for fast improving efficacy and safety, for the good of the field and for the benefit of the patients.

Special Thanks

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Help from The Thalassemia Community
Thank you for helping our Thalassemics by giving them medical support:
Rita Jain, Rajesh Handa, Radhika Gulati, Vijay Wadhwa

If you have important news you want to share with the thalassemia community, let us know. We also encourage people to share stories about their personal experience that may touch other Thalassemics, parents and societies.

For your information:
The below mentioned drugs and equipments are available at Thalassemics India office at subsidized rates:

- Desferal
- Asunra
- Desirox
- Kelfer
- Defrijet
- Pall RC1VAE filter (for 1 unit of blood)
- Pall RC2VAE filter (for 2 units of blood)
- Bio –R filter (for 1 unit of blood)
- Bio –R2 filter (for 2 units of blood)
One of the main complications in patients receiving multiple blood transfusions throughout their lives - including patients with thalassemia major and intermedia in the most severe forms - is iron overload in different organs. Of these, the heart in particular is one of the main organs affected along with the liver. Until the beginning of this decade, heart iron overload was a major cause of mortality and loss of quality of life in thalassemia patients, a reality that started to change in the early 2000s thanks to early diagnosis and improved chelation therapy. The role of magnetic resonance imaging (MRI) was crucial for this change and the role of the exam in early identification of iron overload will be discussed in this document.

**MRI Iron Overload Assessment**

MRI is a special radiological exam which is based uniquely on radio-frequency signal, which means no use of radiation with safety for repeat follow-up. The exam, specifically for iron overload assessment, is done without any contrasts and lasts approximately 10-20 minutes. Iron quantification with MRI can be done in almost any organ but the liver and heart are the usual targets. MRI is currently recommended in practically all clinical guidelines published in different countries around the world with a recommendation to be started between 7-10 years old and be repeated annually or earlier in cases of significant iron overload.

Because iron accumulation occurs without symptoms until it is already in a very advanced state, it is essential that we make an early diagnosis to prevent the arrival of these more serious steps. The first way to assess risk is through the blood ferritin measure: this protein partly reflects the total charge of iron in the body and is constantly elevated, may indicate an increased cardiac risk in the longer term and high iron concentrations in the liver. However, it is important to remember that only a single measure is not able to predict the amount of iron in the heart and, therefore, it alone does not serve to assess heart disease.

Specific cardiac tests can help diagnose heart disease and are routinely applied in patients with thalassemia for this purpose as the electrocardiogram and echocardiogram. The first is the analysis of cardiac electrical conduction while the second looks at the heart using ultrasound techniques. Although useful, changes in these exams occur in later stages of the disease and are not able to tell the clinician the amount of iron already deposited in the heart.

The test that has most proven value for cardiac evaluation in patients with iron overload is the MRI. The technique of resonance was developed in 2001 and in the countries where it has been adopted extensively promoted a significant change in the early diagnosis of myocardial iron overload. MRI measures a variable called $T2^*$. This complicated name is nothing more than the measure of the time (in milliseconds) it takes for the magnetic signal to drop from 100% until it reaches zero. The more iron in a tissue, the faster the signal falls - then the lower the $T2^*$ value, more cardiac iron. The higher the $T2^*$, the lower the iron concentration.

Several studies over the past decade have shown that cardiac $T2^*$ is able to predict which patients have a higher risk of presenting symptoms and arrhythmias with an increased risk of cardiac death. Moreover, the exam also proved effective in monitoring chelation therapy, indicating whether the therapy is effectively removing myocardial iron and protecting the body. But more important than all these aspects, we now know that patients who have $T2^*>20$msec have low risk of death from heart disease or symptoms of disease. On the other hand, patients with $T2^*<10$msec and especially those with $T2^*<6$msec, are more likely to have these problems. By monitoring thalassemia patients routinely, we can identify patients who for some reason are entering these risk areas and intervene before heart disease sets in.

Cardiac MRI technique was recently compared to myocardium biopsies of thalassemia patients who unfortunately died but could make a contribution to help others by allowing the study showing correlations between
the values of the MRI and the direct measurement of cardiac iron. This allowed us today to have a MRI report that gives very precise values of the amount of iron in the myocardium and not just the T2* value.

We also must remember that the procedure for assessment of cardiac iron is also widely used to evaluate the liver and other organs as previously mentioned. This can be performed simultaneously in the same test, with minimum extra time or patient discomfort.

How do we manage patients based on MRI information?

Current treatment of thalassemia relies on regular blood transfusions and iron chelation excess. This allows the thalassemic patient to have a normal life with extensive quality of life and low complication levels. For this to occur, it is important that targeted therapies be based on the degree of iron accumulation in each organ. Hence the importance of an accurate diagnosis, since the estimate of cardiac and liver iron overload from ferritin is limited as mentioned above.

For the heart, when the T2* is above 20msec usually current chelation therapy is maintained since the value indicates that the iron overload is minimal or absent (Figure 1). For values between 10-20msec the degree of overload starts to get a little bigger and this might indicate that probably there is a need to review the current chelation therapy, adherence to treatment and its intensity, modifying it according to specific criteria. If T2* is below 10msec (Figure 2), this value indicates a higher iron overload and chelation therapy should be intensified in some way. It is important to note that the cardiac T2* changes very slowly and over long periods of time: thus, if a patient has an initial low cardiac T2*, it will take from months to years to significantly change this value and MRI monitoring should be routine in these cases.

Figure 1: an example of a heart with normal iron overload in MRI with a T2* of 22.1ms

Figure 2: a severely overloaded heart with T2* of 5.2ms. Note how the liver is also severely affected with very low signal in the image.

Congratulations !

- Congratulations to Swati Tuteja for being an active member of National Service Scheme (NSS). She recently won a medal and a certificate in acknowledgement of her selfless work.
- Sanya Katyal for scoring 85.6% in class 12th CBSE, 2015 Arts Stream.
- Mehak Bhasin for scoring 73% in class 12th CBSE, 2015 Arts Stream.
- Raghav Arora for scoring 76% in class 10th CBSE Board Exam’ 2015
bluebird bio Reports New Beta-thalassemia Major and Severe Sickle Cell Disease Data from HGB-205 Study at EHA

• Patient with Severe Sickle Cell Disease Producing 45% Anti-sickling Hemoglobin at Six Months and Has Been Free of Transfusions for More Than Three Months
• Patients with Beta-thalassemia Major Remain Transfusion-Independent at 16 and 14 Months, Respectively
• First Patient with Severe Sickle Cell Disease Infused in HGB-206 Study
• Investor Conference Call Scheduled for June 15, 2015 at 8:00 a.m. ET

VIENNA, Austria and CAMBRIDGE, Mass., June 13, 2015 – bluebird bio, Inc. (Nasdaq: BLUE), a clinical-stage company committed to developing potentially transformative gene therapies for severe genetic and rare diseases and T cell-based immunotherapies, today announced long-term follow up of two patients with beta-thalassemia major and early safety and efficacy data in the first patient with severe sickle cell disease (SCD) treated with LentiGlobin BB305 product candidate in the HGB-205 study. These data were presented today at the 20th Congress of the European Hematology Association (EHA) in Vienna, Austria by Marina Cavazzana, M.D., Ph.D., lead investigator of the HGB-205 study and professor of hematology at Paris Descartes University, head of the department of Biotherapy Hospital, the clinical research INSERM center of Biotherapy at Necker Enfants Malades, (Assistance Publique-Hôpitaux de Paris) and the Lymphohematopoiesis Laboratory, Institute of Genetic Diseases, Imagine, Paris, France.

Key findings
• Beta-thalassemia:
  o As of May 2015, Subjects 1201 and 1202 with beta-thalassemia major remained transfusion-independent for 16 and 14 months, respectively, with persistent stable expression of HbAT87Q. Neither has experienced a LentiGlobin-related adverse event.

• Sickle Cell Disease:
  o The proportion of anti-sickling hemoglobin being produced by the first-ever patient with severe SCD treated with gene therapy (Subject 1204) is rising steadily and accounted for 45% of all hemoglobin production (40% HbAT87Q + 5% HbF) at the patient’s six-month visit post-drug product infusion; this is above the 30% threshold expected to potentially achieve a disease-modifying clinical effect.
  o As of May 2015, Subject 1204 had been free of transfusions for more than three months without complications or hospitalizations for SCDs related events post-transplant, and with improvement in hemolysis markers “These data are promising for patients living with beta-thalassemia major and severe sickle cell disease, two devastating, genetically-based hematologic diseases that have a profound impact on both quality of life and life expectancy,” said Professor Cavazzana. “The steady rise and high-level of HbAT87Q production in our patient with severe sickle cell disease is cause for optimism as we expect levels of anti-sickling hemoglobin of 30 percent or more could significantly improve and potentially eliminate the serious and life-threatening complications associated with sickle cell disease.” bluebird bio also announced that the first patient with severe SCD has been infused in the HGB-206 U.S.-based clinical study at the National Institutes of Health Clinical Center in Bethesda, Maryland. HGB-206 is a Phase 1, U.S.-based clinical trial evaluating the safety and efficacy of bluebird bio’s LentiGlobin BB305 product candidate in subjects with severe SCD. “Today’s data further demonstrate the potentially transformative effects of gene therapy for the treatment of beta-hemoglobinopathies and support our global regulatory strategy for LentiGlobin in beta-
Patients with beta-thalassemia major are now extending to the first treated patient with severe sickle cell disease. With the treatment of the first patient with severe sickle cell disease in the HGB-206 study, we look forward to gaining increasing clarity on the potential clinical benefit of LentiGlobin for patients with severe sickle cell disease.

HGB-205 Study Data

HGB-205 is an ongoing, open-label, single-center Phase 1/2 study designed to evaluate the safety and efficacy of LentiGlobin BB305 product candidate for the treatment of patients with beta-thalassemia major and severe SCD. As of May 2015, two patients with beta-thalassemia major and one patient with severe SCD have undergone infusion with LentiGlobin BB305 product candidate in this study.

- Patients with beta-thalassemia major demonstrated continued transfusion independence: The data presented today are an update on those initially presented at EHA in June 2014 and demonstrate a long-term stable and durable response. As reported at ASH in December 2014, Subjects 1201 and 1202 achieved rapid transfusion independence with normal hemoglobin levels. As of May 2015, Subjects 1201 and 1202 remained transfusion independent for 16 and 14 months, respectively.

- Early efficacy data is promising in the first subject with severe SCD treated with gene therapy: The production of anti-sickling hemoglobin is consistent with a level expected to potentially achieve a disease-modifying clinical effect in Subject 1204. At the six-month visit post-drug product infusion, the proportion of anti-sickling hemoglobin (HbAT87Q + HbF) accounted for 45% of all hemoglobin production (40% HbAT87Q + 5% HbF). The patient's vector copy number in peripheral blood leukocytes was 2.9.

- Subject 1204 had his last red blood cell (RBC) transfusion at day 88. Since his infusion with LentiGlobin, this patient has had no hospitalizations, and has demonstrated improvements in markers of hemolysis, including normalization of reticulocyte count (from 283.3 x 109/L to 131.7 x 109/L) and lactate dehydrogenase (LDH) (from 266 U/L to 254 U/L).

In the HGB-205 study, treatment with our LentiGlobin BB305 product candidate has been well-tolerated to-date, with no LentiGlobin-related adverse events observed. All of the adverse events observed are consistent with myeloablative conditioning.

All three subjects successfully engrafted and inserted site analyses (ISAs) demonstrate highly polyclonal reconstitution without clonal dominance.

About Beta-thalassemia

Beta-thalassemia is an inherited blood disease that can cause severe anemia. Patients with beta-thalassemia cannot make enough of the beta-globin part of hemoglobin, the protein used by red blood cells to carry oxygen throughout the body. Approximately 60,000 children are born with a serious form of the disease each year, making it one of the most common genetic diseases in the world. In its most severe form, beta-thalassemia is fatal if not treated.

Treating beta-thalassemia includes frequent and lifelong blood transfusions, which deliver red blood cells to the body to correct the anemia. However, blood transfusions also cause excess iron to build up in the body, which can damage organs and cause additional issues, such as abdominal pain, weakness, fatigue, joint pain, endocrine dysfunction, liver cirrhosis and heart failure. Patients who receive ongoing blood transfusions must also receive...
treatment to remove the excess iron. The only currently available curative treatment option for beta-thalassemia is allogeneic hematopoietic stem cell transplant. However, these transplants are typically offered to pediatric patients with matched related donors (occurring in less than 25 percent of all cases), due to the significant risk of transplant-related morbidity and mortality.

About Sickle Cell Disease
Sickle cell disease (SCD) is an inherited blood disorder resulting from a mutation in the beta-globin gene that causes polymerization of hemoglobin proteins and abnormal red blood cell function. The symptoms of SCD include anemia, vaso-occlusive crises (a painful complication caused by obstruction of the blood vessels), infections, stroke, and progressive end-organ damage leading to overall poor quality of life and early death in a large subset of patients. The global incidence of SCD is estimated to be 300,000 births annually, and the global prevalence of the disease is estimated to be about 25 million.

Patients with severe SCD typically receive chronic blood transfusion regimens or hydroxyurea. Chronic transfusions for SCD introduce the risk of iron overload, which over time contributes to mortality through iron-associated heart and liver toxicity, and patients must adhere to daily iron chelation regimens. While hydroxyurea has been shown to significantly reduce the burden of vaso-occlusive crisis and related complications, it does not eliminate them. The only potentially curative therapy is allogeneic hematopoietic stem cell transplant (HSCT). Because of the significant morbidity and mortality risks associated with transplants, they are usually offered only to patients who have sibling matched donors and only 10 percent of SCD patients of African descent are able to find such donors.

About the HGB-205 Study
HGB-205 is an ongoing, open-label Phase 1/2 study designed to evaluate the safety and efficacy of bluebird bio’s LentiGlobin BB305 product candidate in the treatment of subjects with beta-thalassemia major and severe sickle cell disease (SCD). The study is designed to enroll up to seven subjects who will be followed to evaluate safety and transfusion requirements post-transplant. Among patients with SCD only, efficacy will also be measured based on the number of vaso-occlusive crises or acute chest syndrome events. For more information on the HGB-205 study, please visit clinicaltrials.gov using identifier NCT02151526.

About the HGB-206 Study
HGB-206 is an ongoing, open-label Phase 1 study designed to evaluate the safety and efficacy of LentiGlobin BB305 product candidate in the treatment of subjects with severe sickle cell disease (SCD). The study is designed to enroll up to eight subjects to evaluate safety and efficacy as measured by changes in red cell function tests and hemolysis markers, as well as clinical events secondary to SCD, including vaso-occlusive crises or acute chest syndrome events. For information on the HGB-206 study, please visit clinicaltrials.gov using the identifier NCT02140554.

About bluebird bio, Inc.
With its lentiviral-based gene therapy and gene editing capabilities, bluebird bio has built an integrated product platform with broad potential application to severe genetic diseases and T cell-based immunotherapy. bluebird bio’s clinical programs include Lenti-D™, currently in a Phase 2/3 study, called the Starbeam Study, for the treatment of childhood cerebral adrenoleukodystrophy, and LentiGlobin®, currently in three clinical studies: a global Phase 1/2 study, called the Northstar Study, for the treatment of beta-thalassemia major; a single-center Phase 1/2 study in France (HGB-205) for the treatment of beta-thalassemia major or severe sickle cell disease; and a separate U.S. Phase 1 study for the treatment of sickle cell disease (HGB-206). bluebird bio also has ongoing preclinical CAR T immunotherapy programs, as well as discovery research programs utilizing megTALs/homing endonuclease gene editing technologies.

bluebird bio has operations in Cambridge, Massachusetts, Seattle, Washington, and Paris, France. For more information, please visit www.bluebirdbio.com.

About the Institute of Genetic Diseases -- Imagine
Imagine is a research and innovative healthcare institute of a new type, bringing together researchers, doctors and patients, with a common goal: to cure genetic diseases. This new Institute is housed in a 19,000 m² building located on the campus of the Necker Enfants Malades Hospital, and brings together over 850 researchers, doctors and healthcare personnel, with an innovative vision: gathering all those concerned in the treatment of genetic diseases to create synergy that encourages transfer of knowledge, to speed up the discovery of new treatments and diagnoses to meet the expectations of the patients and their families.

About the AP-HP
AP-HP, Greater Paris University Hospital, is a care provider known worldwide with its 38 hospitals and the largest university medical center in France and in Europe. Each year, AP-HP welcomes 7 million patients, takes care of more than 1.1 million emergencies and makes more than 5 million outpatient visits. AP-HP is the largest employer in Paris Region with 95,000 people -- doctors, researchers, paramedical, administrative staff and workers.
Forward-Looking Statements

This release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the potential efficacy and safety of the Company’s LentiGlobin product candidate, in subjects with beta thalassemia major and severe sickle cell disease, including statements concerning the reduced or eliminated need for transfusion support for the study subjects and the potential reduction in symptoms of severe sickle cell disease, statements concerning the Company’s future plans with respect to LentiGlobin and its other product candidates and statements concerning the HGB-206 clinical trial in severe sickle cell disease. It should be noted that the data for LentiGlobin announced from the HGB-205 study at the EHA Congress are preliminary in nature and the Northstar, HGB-205 and HGB-206 studies LentiGlobin are not completed. There is limited data concerning long-term safety and efficacy following treatment with LentiGlobin drug product. These data may not continue for these subjects or be repeated or observed in ongoing or future studies involving our LentiGlobin product candidate, including the HGB-205 Study, the Northstar Study or the HGB-206 study in severe sickle cell disease. It is possible that subjects for whom transfusion support has been reduced or eliminated may receive transfusion support in the future and that the subject with severe sickle cell disease may experience serious symptoms of the disease. Any forward-looking statements are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to, the risk that the preliminary results from our clinical trials will not continue or be repeated in our ongoing clinical trials, the risk that previously conducted studies involving similar product candidates will not be repeated or observed in ongoing or future studies involving current product candidates, the risk of cessation or delay of any of the ongoing or planned clinical studies and/or our development of our product candidates, the risk of a delay in the enrollment of patients in the Company’s clinical studies, the risk that our collaboration with Celgene will not continue or will not be successful, and the risk that any one or more of our product candidates will not be successfully developed and commercialized. For a discussion of other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section entitled “Risk Factors” in our most recent quarterly report on Form 10-Q, as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and bluebird bio undertakes no duty to update this information unless required by law.

Investor Relations:
Manisha Pai
bluebird bio, Inc. (617) 245-2107
mpai@bluebirdbio.com

Media: Dan Budwick
Pure Communications, Inc.
(973) 271-6085

Upcoming Events

- 6th International Conference on Thalassemia –TBA
- Annual General Meeting –TBA
- Thalassemia Checkup Clinics in Delhi and other parts of the country- TBA

If you wish to donate, please visit our website www.thalassemicsindia.org for online donations, else you can pay by cheque favoring “Thalassemics India”. Your contribution will be used by us in helping BMT cases and also for those who cannot afford chelation drugs and filters on their own.
Thalassemics India’s Activities

Visit to ISKCON Temple
A group of 65 thalassemia children and their parents were taken to the Iskcon temple New Delhi for (Gita Saar Utsav). The 2 hour programme at Iskcon temple auditorium included movie, games & gifts for all. The visit was organized in association with ISKCON.

Annual Picnic / Outing:
On May 20th, fifty Thalassemics and their parents were taken to Kingdom of Dreams, Gurgaon to see the show ‘Jhumroo”. The second batch was taken to KOD on June 17th. After the show the children are given smart cards for entry to the culture gully and snacks.

Towards awareness
• A talk was given by Mrs. Poonam Anand, executive member of Thalassemics India at RPVV, Civil Lines on 13th May 2015. The talk was attended by 170 lecturers of biology subject, from Directorate of Education NDMC & Govt. aided schools.
• Messages about Thalassemia for public awareness were given by the society on radio during the first week of May.
• Keeping the momentum going, thalassemia information material was distributed in the monthly meetings of AOGD. Material is also being kept in the clinics of Gynaecologists & Obstetricians throughout the city of Delhi.
• Additional material in the form of book marks, leaflets, posters and pamphlets on thalassemia awareness is printed for free distribution in hospitals and private clinics.
• As part of Thalassemics India awareness campaign, Dr. V.K. Khanna was invited by Doordarshan for a Live Chat “Good Evening India” on 16th June.

MRI meeting at Apollo Hospital, New Delhi
The meeting held at Apollo Hospital under the banner “MRI Tour 2015 India” was organized by Thalassemics India with the support of Novartis Oncology under the chair : Dr. Juliano Lara Fernandes.

Dr. Juliano Lara Fernandes has developed an excel sheet that can enable us to use any MRI equipment and calculate the iron overload in heart and liver. This tool has been validated. In this one day workshop, Dr. Fernandes shared in detail the use of this tool and also give a practical demonstration.

Presentations were made on Liver & heart loading and unloading, Clinical complications due to IOL, Cardiac/liver IOL assessment by MRI & Fundamentals in MRI image interpretation. Practical sessions were held followed by Q & A. The meeting was attended by 30 doctors and technicians from Delhi and from other parts of the Country.
**Fund Raiser event: 18th April**

Thalassemics India hosted an evening of Sufi music with Sonam Kalra and the Sufi Gospel Project. This was a fund raiser event to collect funds to support the treatment of under privileged Thalassemia children. The function was held at Siri fort Auditorium from 7.30 pm onwards on April 18th. This evening saw an audience of around 350 people. We thank Sonam Kalra and the troupe for giving a mesmerizing evening.

**Summer Funk**

Thalassemics India is grateful to Victory Arts Foundation for having given our thalassemia children an opportunity to perform in the Summer Funk 2015 at Siri fort Auditorium on 13th June. These 10 Dancers had given a stunning performance even on 8th May International Thalassemia Day.
bluebird bio is developing next-generation products based on the transformative potential of gene therapy to treat patients with severe genetic and rare diseases.

TRANSFORMING THE LIVES OF PATIENTS WITH SEVERE GENETIC AND RARE DISEASES

Learn more about bluebird bio at www.bluebirdbio.com
Contact us about participation in our clinical studies at clinicaltrials@bluebirdbio.com
Why patients in India need FerriScan® - the gold standard LIC measurement

Measurement of liver iron concentration (LIC) by MRI provides a more reliable and accurate indication of patients’ iron loading than can be assessed from serum ferritin, and spares them from an invasive liver biopsy, which can be both painful and also susceptible to sampling errors. Iron generally loads in the liver before reaching a critical threshold after which loading in other organs such as the heart occurs. As such it is extremely important to monitor and manage LIC to prevent complications of iron overload.

There are a number of different MRI methodologies available for measuring LIC, the most common of which are FerriScan R2-MRI or liver T2*.

FerriScan R2-MRI is accepted in the global medical community as the gold standard in LIC measurement. FerriScan is the only MRI liver iron measurement method that has been calibrated against biopsy (in 105 patients) on multiple scanners and has been successfully validated against biopsy in an independent cohort of 233 patients on different scanner makes and models.

FerriScan has gained clearance by regulatory agencies worldwide, and in addition to its use in clinical practice, is used by pharmaceutical companies researching and developing iron chelation treatments. FerriScan has recently been mandated for use by the FDA in treatment of patients with non-transfusion-dependent thalassaemia; as no other MRI method for LIC measurement was considered reliable enough.

In Italy a group of 12 hospitals has formed a network called LICNET. They evaluated the different MRI measurement technologies and plan to expand their network using FerriScan, having determined its superiority.

How FerriScan works

FerriScan images are acquired and transmitted for analysis from MRI centres that have undergone a rigorous validation process. Currently around 920 MRI centres in over 30 countries worldwide are accredited FerriScan sites. After the image data is confirmed to adhere to the FerriScan requirements, the patented FerriScan analysis technique is applied in order to produce a colour-coded map of the iron loading across the liver and a mean LIC. Over 28,000 such analyses have been performed to date. All analyses are performed in accordance with quality assured processes to ensure consistent and highly accurate results.

MRI centres are supported in the FerriScan validation process, which demands good technical competency and attention to detail from MRI centre technicians. It is through adherence to such strict standards that FerriScan has attained its place as the gold standard in LIC measurement and is trusted by regulatory bodies, clinicians and pharmaceutical companies worldwide. Other methods lack this standardisation and do not have multi-centre, multi-scanner validation.

Other MRI-based methods

Liver T2* measurements are used at some centres, some of which claim to have been be validated against, or show a strong correlation to, FerriScan. These methods are inherently limited by having being calibrated against biopsy on a single scanner, and would therefore not be reliably transferrable to different makes and models of scanner. Additionally there is no standardisation of data acquisition parameters or analysis techniques between centres and often no checks in place to ensure that data acquisition parameters remain consistent over time. A ‘strong correlation’ does not mean that another method can reliably give the same results as a FerriScan in clinical practice. These failings have limited the extent to which many T2* techniques have been able to achieve regulatory clearance.

The graph below shows a range of LIC results produced by different T2* calibrations.

Research performed by leading authorities such as the Whittington Hospital in London has shown that liver T2* results can very seriously underestimate iron loading at the higher end of the LIC range, resulting in inadequate chelation. Liver T2* measurement can also be impacted by fibrosis or cirrhosis whereas...
FerriScan is not affected by these factors.

Advances for FerriScan in India

Resonance Health, the company providing FerriScan, has had a long and close partnership with the Thalassaemia International Federation (TIF) internationally. The TIF Board recognize the superior accuracy and reliability that FerriScan offers over other services, and this is highlighted in TIF Guidelines. Whilst FerriScan is very cost effective compared to an invasive liver biopsy it is generally more expensive than other, less reliable MRI methodologies. This is because of the need to ensure global standardisation of data acquisition, and the quality controls around its analysis and reporting.

Resonance Health is committed to overcoming these funding barriers and currently has a particular focus on India. Projects are being initiated with TIF and country patient societies that we hope will ultimately gain funding by Ministries of Health. A new project is currently under discussion to provide access for larger numbers of patients in countries such as India where there is real need. Support from the clinical and patient community is vital to the success of these projects.

In India, Resonance Health has worked closely with a pharmaceutical company to provide access to FerriScan at two centres. We have been able to provide the service for a small number of patients through this project. We hope that this service will be expanded to other centres in the future, until such time as broader funding can be established to make FerriScan available to all those in need.

Medical Books are available at Thalassemics India office.
Hard copies can be ordered directly from Thalassemics India distributed free of cost (only one copy per publication).

-Wendy Wardell
Public Relations Consultant

Life is a stricter Teacher than a Teacher, Because a Teacher teaches the lesson first and then keeps Exam, But Life keeps Exam first then teaches the Lesson.
Girl Child to a Person

There was a cry from the cradle,
There was a sweet girl in the cradle.

    Years rolled by,
    She became woman.
    From white hair to grey hair.
    From one feet child,
    To five and half feet woman.
    From birthday dress to clad in saree.
    Sitting on the pail of house,
    She thought how all these happened.

    Image of a crawling child,
    Came in front of her eyes.
The crawling child grew into a girl in frock,
    The frocked turned into a uniform.
    She was thankful for her form,
    In the school with lots of norm.
    She was thankful to her parents,
    Her teacher for grant of education.

    The intelligent girl in college,
    Earning lots of knowledge.
A young woman in the office,
    A married woman in the bliss.
    All came before her,
A responsible wife and a loving mother,
    A responsible officer together,
Has made a person from a girl.

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A Japanese Fully Automated 3 Part Differential Hematology Analyzer

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Simplicity Made for You

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Screening for inflammation
Distinct Neutrophil population provides clinical data useful for better patient management

Screening for anaemia
Both RDW-SD and RDW-CV combined with MCV can set criteria for differentiation of iron deficiency anaemia and beta thalassemia.

Screening for the cause of thrombocytopenia
PDW, P-LCR and MPV have sufficient sensitivity and specificity in the diagnosis of immune thrombocytopenia and idiopathic thrombocytopenic purpura (ITP)*

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SNCS ensures real time QC assessment.

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#### We welcome the following new members

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<td>Vinod Kamdar</td>
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TIF Board of Directors meeting, held in London on 2nd and 3rd of May, 2015. The meeting was attended by Mrs. Shobha Tuli on behalf of Thalassemics India. A no. of items were discussed as per the agenda. After a brief address by the President, discussions were held and reports were shared on the final accounts, approval of the minutes of last board meeting, list of new members for approval, executive directors’ report, next TIF conference, Pan Asian Conference, TIF delegation visits and the Indian project. Talks & discussions were held on Gene Therapy, MRI & Ferriscan as part of the TIF Board meeting.

Thalassemics India would like to thank Thalassaemia International Federation for supporting the participation of 4 candidates from India to attend the 2nd Pan-Asian Conference, to be held in Hanoi, Vietnam.

For more information contact: www.thalassaemia.org.cy
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Leukocyte depletion filters for whole blood, red cell concentrate or platelet concentrate

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Leukoreduced transfusions reduced post-operative infections by about 10% (from 33% to 23%) in Surgical Patients.

After implementation of universal leukoreduction in transfused patients, the Line Related Infections were seen to reduce by 35%.

For patients undergoing Bone Marrow Transplantation filtration of the blood products was effective for the prevention of transfusion-associated CMV infection.

LR blood transfusion in Cardiac Surgery Patients reduced mortality rates by half.

Patients given LR blood had a much lower incidence of Bacterial Contamination as compared to those given non-LR blood.
Haemonetics Purecell™ RC
High efficiency leucocyte removal filter for blood transfusion

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- High red cell recovery
- Minimal filter hold-up volume
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- For standard or rapid flow applications

The blood filters business of PALL® is now a part of Haemonetics
Activity report from Punjab by Dr. Praveen Sobti

On 7.5.2015, a voluntary blood donation camp was organised at Dayanand Medical College and Hospital Ludhiana.

At Christian Medical College Ludhiana a CME on Comprehensive Care of Thalassemics was conducted, entitled from “Womb to Well-being” Special emphasis was laid on prevention and the Chief Guest Sh. Rajat Agarwal the Deputy Commissioner was apprised of all aspects of thalassemia care in Punjab and the facilities available in CMCH. He has promised to look into the matter more closely.

A voluntary blood donation drive for medical students was also organised under the patronage of Punjab Thalassemics Welfare Society, wherein medical students, registrars and faculty donated 300 units of blood.

A trainers group has been formed in Punjab, with faculty from PGI, DMCH and CMCH with the aim to disseminate the knowledge on management of thalassemia according to TIF guidelines. The first workshop was held in the medical college Faridkote. The next one is scheduled for Barnala next month.

News Across India

Report from Mumbai by Sangeeta Wadhwa

International Thalassaemia day was celebrated by Shri Hashu Advani Memorial Foundation on 4th May 2015, at Sargam Water Resort, Mumbai. 230 Participants were there. The trustees of the foundation distributed gifts and medicines to the patients.

A blood donation camp and health Check up camp was also organized at VES IT College Chembur on 22nd February on the occasion of the founder trustee Shri Hashu Advani Jis (commemorate) Birth Anniversary. Day

On 24th March a blood donation camp, a health Checkup clinic & Thalassaemia Screening camp was held at Management College, Mumbai. An awareness talk on “Blood disorder and Healthy life” was also arranged for Students and staff of the college.

Report from Nasik by Dr Sangeeta Lodha

Lecture addressing by Dr. Sangeeta Lodha (pediatrician) to younger future doctors. Thalassaemia Screening programme at Arifiant Hospital, Mumbai under the banner of Mahindra & Mahindra Group.

Report from Pune by Dr. Nita Munshi

Thalassemia Society celebrated International Thalassaemia Day on 8th May. Ruby Hall Clinic had organized a walkathon for parents and patients along with Committee members of Thalassaemia Society of Pune Chapter. All children were given hats and red T shirts. Placards of “Prevent Thalassaemia” were carried by all. Society distributed pamphlets to general public about Thalassaemia What is it and how to prevent it.

They had a magic show and puppet show for kids in the auditorium. Jackie Shroff was the guest of honour who obliged the kids with photos,
autographs, and a sweet talk. Overall the children had a great time. They were then given lunch and gifts.

Report from Ulhasnagar by Thadharam Tolani.

Thalassemia detection camp at Jai Bab Hall, Ulhasnagar through Thyocare, organized by Thadharam Tolani.

Report from Ajmer by Ishwar Parwani.

Kozhikode. Blood Patients Protection Council (BPPC) has observed World Sickle Cell Day and World Blood Donor day on 14th June 2015 at Regional Science Center & Planetarium calicut. In this part various programs like honoring blood Donors, distribution of cash awards to blood disorder patient pupils, Sickle cell patient’s session, distribution of education kits and poster making contest for blood disorder students in theme of “Thank You for saving My Life” were organized. As a main program of the celebration, blood disorder patients like Thalassemia, Sickle cell Anemia, Hemophilia and Leukemia honored all the blood donors by offering respect to 59 time blood donor philanthropist Dr. V.P. Sasidharan, Head, department of Transfusion medicines, Medical college Hospital calicut. K.M. Sunil, Education Officer, Regional Science Center distributed the P. Anshif memorial cash awards to blood disorder patients those who were passed SSLC and BA Examination this year. Dr. Mehul Mahesh, past vice president of JCI Calicut chapter distributed the educational kits to the all participated families. The program demanded to the state and central Government to set up a full fledged Hematology Center at Medical college hospital, Calicut and also demanded to provide blood component like Cryoprecipitate and Leucocyte filtered blood without considering their age or financial background. Council also repeated its long standing demands rised in 2011 World Sickle day. Council felicitated F.S. Hasna, a Thalassemia patient for passing the BA examination. Hasna is the first thalassemia Major patient passed BA examination in Kerala.

A poster making contest also conducted in the wake of World Blood Donor day and subject of this year’s theme “Thank You for Saving My Life”. Shamal A.C, Navya C and Geethika E, won the first, second and third prizes respectively in the contest. Rail concession certificate for Sickle Cell Disease, Hemophilia and thalassemia also distributed.
Once-daily dosing with Deferasirox: >200mg/kg/d provided sustained reduction in LPI levels in these heavily iron overloaded β-thalassemia patients, supporting 28-day production from LP1.

The most frequent reactions reported during chronic treatment with Deferasirox in adult and pediatric patients include gastrointestinal disturbances in about 26% of patients (mainly nausea, vomiting, diarrhea, or abdominal pain), and skin rash in about 15% of patients. These reactions are dose-dependent, mostly mild to moderate, generally transient, and mostly resolve within 11 days of treatment. No ANM neuroprogresion in skin, cardiovascular, or gastrointestinal (mostly related to the normolage, occurs in about 35% of patients. These are dose-dependent, often resolve spontaneously and can sometimes be mitigated by reducing the dose.

Pediatric Patients: identified as 3 years and above.


Basic Success Statement

ASUNRA: prevention, treatment, and cure of iron overload, the leading cause of organ damage and death in iron-loading anemias. Once-daily dosing with Deferasirox: >200mg/kg/d provided sustained reduction in LPI levels in these heavily iron overloaded β-thalassemia patients, supporting 28-day production from LP1.

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1. Deferasirox reduces unregulated tissue iron loading and prevents further end-organ damage in β-thalassemia patients. 2. A novel once a day oral iron chelator. 3. They are in your heart. They are in your liver. Live as you were take care of them. 4. Asunra® deferasirox 10mg/kg/day tablets. 5. They are in your heart. They are in your liver. Live as you were take care of them.