



PSOD kicks-off campaign for 500,000 signatures

WITH the goal of reaching half a million signatures in support of Senate Bill 3087, PSOD launched its petition campaign via CARE 2 Petition (www.petitionsite.com), an online petition site for social change.

The campaign's objective is to increase the awareness on rare diseases among the Filipinos here and abroad, get their support, and rally them in pushing the lawmakers to pass SB 3087 which seeks to establish a system that will help ensure the early diagnosis and treatment of rare diseases in the Philippines

PSOD aims to reach the target number of signatures by February 2010 as it joins the World Rare Diseases Week celebration.

To sign the petition, visit:
<http://www.thepetitionsite.com/3/please-endorse-sb-3087-the-rare-diseases-act-of-philippines>



1. sign 2. confirm 3. share 4. thank you!

**PLEASE ENDORSE SB 3087,
The Rare Diseases Act of
the Philippines**

Target: General Public and
Rare Disease Advocates

Sponsored by: Philippine Society for
Orphan Disorders, Inc. (PSOD)

signature
goal: 500,000



sign petition!

Angara Bill promotes awareness for rare disorders

FILIPINO families with children afflicted with rare disorders and the Philippine Society for Orphan Disorders, Inc. (PSOD), got a big boost when Senator Edgardo Angara announced last April 2009 his support for the "Right to Life" by filing Senate Bill 3087 or the Rare Disorder Act of the Philippines. He made his announcement during the 10th year anniversary celebration of the Institute of Human Genetics held at the Rigodon Hall of the Manila Hotel last February 25, 2009.

The bill, as proposed by Senator Angara, is intended to promote greater awareness for rare or orphan disorders which afflict Filipino children across different socio-economic classes, and determine ways of funding support, including fiscal regulation and providing incentives for medical research and aid.

Dr. Lulu Bravo, currently the head of the Department of Health's National Institutes of Health, applauded Senator Angara's support for the advocacy and committed her department's support for PSOD's goals, specifically in research, policy formulation and medical training.

Genzyme Corporation's Dr. Patrick Granjard, Southeast Asia regional director, and Dr. Margarita Jimenez, its Asia Pacific medical director, shared updates on medical treatments available for rare disorders during the media forum. Genzyme Corporation is an international biotech company based in Boston.

Thru a video clip, celebrity Carminia Villarroel, TV actress/host and a mother, expressed her support for PSOD and encouraged the public to help in the advocacy for awareness and support for rare disorders.

Rare disorders also known as "orphan diseases" are long-standing, life threatening, progressive and disabling which require multi-disciplinary care and afflicts babies in all social-economic classes. These include such diseases as maple syrup urine disease, Gaucher disease, Prader Willi syndrome, Pompe disease, Galactosemia, Fabry disease and MPS I/II, among others.



Senator Angara, a staunch supporter of the scientific community, reaffirmed his commitment to support the advances in human genetics and the right to life by sponsoring Senate Bill 3087 or the Rare Disorder Act of the Philippines.

Treatments for these disorders are available but are expensive, often beyond the reach of families who have children afflicted with these.

Since the PSOD's organization, its founding chairman, Dr. Carmencita Padilla and concurrently the head of the Institute of Human Genetics or IHG and its president, Cynthia Magdaraog, the mother of a Pompe patient, have worked tirelessly in order to promote more public awareness and funding for patients with rare disorders.

PSOD aims to increase public awareness on rare disorders in the country, establish and develop a nationwide registry, promote and support relevant and timely research for a better understanding and treatment; participate in policy formulation, develop and strengthen the relation among involved institutions, develop and provide training for health professionals, and mobilize resources and funds for patients' needs.

For more information on PSOD, log on to www.psod.org.ph or email info@psod.org.ph or call telefax no. +632 725-6519.#

About The Philippines Society for Orphan Disorders (PSOD)

People Behind PSOD

The people behind the birth of the Society are two parents, two geneticists, an endocrinologist, a neonatologist, an occupational therapist, a lawyer, and a priest/medical doctor.

Carmencita D. Padilla, MD, MAHPS

Founding Chairman
Geneticist, Pediatrics
Director, Institute of Human Genetics,
National Institute of Health,
University of the Philippines-Manila

Cynthia K. Magdaraog

President
Parent of Pompe Disease Patient/
Businesswoman

Elpidio M. Paras

Vice President
Grandparent of Maple Syrup Urine Disease patient
Businessman

Sylvia C. Estrada, MD

Secretary
Pediatric Endocrinologist and Metabolic Physician
for Newborn Screening Follow-up Program,
Institute of Human Genetics,
National Institute of Health,
University of the Philippines-Manila

Amelia R. Fernandez, MD

Treasurer
Neonatologist
Former Dean of the College of Medicine,
University of the Philippines

BOARD MEMBERS

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Prefect of Health, Phil. Province Society of Jesus
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University

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Molecular Geneticist / Pediatrics
Program Coordinator, Molecular Genetics Unit,
Institute of Human Genetics,
National Institute of Health,
University of the Philippines-Manila

Cristina de Leon-Hinlo

Occupational Therapist
Businesswoman, Therapy Works

The Philippine Society for Orphan Disorders, Inc. serves as a central network for the advocacy and effective coordination of all viable efforts to sustain a better quality of life for the individuals with orphan or rare disorders in the Philippines.

Filipino patients born with rare disorders are “orphaned” by society. They suffer from social abandonment because of lack of existing network of support to aid them. Medical help is elusive under the conditions of the country’s health priority.

The nature of their illness is hardly known due to lack of information and only a few medical professionals, particularly in the country, are aware of these disorders and know how to diagnose and address these conditions.

Over several decades research and clinical trials worldwide have been very slow due to only a handful of known cases. However in recent past, the tireless efforts and the admirable dedication of medical researchers, professionals and drug companies in developed countries have resulted in breakthrough therapy and hopefully, a possible lifelong cure for a growing number of the rare disorders.

In the Philippines, over the past fifteen years, a handful of Philippine medical professionals from the University of the Philippines, National Institute of Health, Institute of Human Genetics have been attending to these patients, dedicating themselves to create awareness

in the country, and continue to undertake clinical trials to contribute to the body of world knowledge that would lead to improving the health of Filipino patients with rare disorders.

Since 1991 these dedicated doctors have been relentlessly sourcing the much needed funds from foreign and local donors, as well as arrange with drug companies to make available under compassionate use the special food formulas and enzyme replacement therapies crucial to sustaining and improving the health condition of patients.

PSOD’s Objectives

To increase public awareness about rare disorders in the Philippines;

To develop and establish a nationwide registry of relevant and material statistical information, medications, drug trials and all other pertinent information about orphan disorders;

To assist patients and their families, family support groups, doctors and researchers in the management of these conditions;

To promote and support relevant and timely researches for a better understanding and treatment of orphan disorders;

To participate in policy formulation, advocacy and legislations of national and international relevance about orphan disorders;

To develop and strengthen the relationship among institutions that are involved in the care of individuals with orphan disorders;

To develop and provide training for parents, families, health professionals, paramedical personnel and community health workers on the care of patients with orphan disorders;

To develop member’s core competencies in the management of orphan disorders; and

To be financially self-reliant and to mobilize resources in the pursuit of organizational objectives.

Because the nature of rare disorders are long standing, life threatening, progressive, and require multidisciplinary care, available therapies and food supplements likewise require life long administration. Until such time further breakthroughs happen (as we are very hopeful it will), without help from private sector, treatment is elusive for the patients due to its prohibitive cost and accessibility.

With increasing number of patients requiring huge amounts of funds, in June of 2006, The Philippine Society for Orphan Disorders, Inc. was organized to continue the efforts of these doctors to ensure sustainability of medical and financial support of patients with rare disorders.

PSOD is non-stock, non-profit organization. Primary sources of funding include contributions, and revenues

from the sale of collateral materials promoting awareness such as t-shirts, keychains, all-occasion cards, etc.#

Care for Rare

The official newsletter of the
PHILIPPINE SOCIETY FOR ORPHAN DISORDERS, INC.

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PSOD Patient Welfare Activities

2007-JUNE 2009

July 2007

A medical mission was conducted for patients with Adrenoleukodystrophy (ALD), Gaucher Disease, Methyl Malonic Aciduria (MMA), Mucopolysaccharidoses (MPS Hunter Syndrome), Carbamyl Phosphate Synthase (CPS) Deficiency, Phenylketonuria (PKU), Maple Syrup Urine Disorder (MSUD), and Pompe Disease. A parent support orientation was also conducted as part of the one-day event aimed at providing the parents with knowledge on the rare diseases, basic care and occupational therapy of their children for daily living, and some tips on business opportunities. The medical mission and parent support orientation were joint projects of PSOD and the Institute of Human Genetics of the National Institutes of Health, University of the Philippines Manila.



August 2007

Thru the kindness and substantial donation of Zonta Metro Ortigas Chapter, headed by its then past president Ms. Malu Cortez and then current president Lizette Lim, PSOD was able to purchase 12 cases SHS-0876-1 MSUD ANALOG 400G as reserve MSUD formula which patients can buy at cost during emergency cases at the Institute of Human Genetics, NIH, UP Manila. Part of the purchased MSUD formula was immediately used to help save the lives of four (4) patients who were then in the intensive care unit of the Philippine General Hospital (PGH).



October 2007

Donation of six (6) wheelchairs to the following patients: four (4) with MSUD, one (1) with CPS and one with MPS Hunter. The wheelchairs were courtesy of Congressman Ed Zialcita of Paranaque. They were turned over to PSOD thru Ms. Connie Zialcita-Trinos, president of the Rotary Club of Paranaque Southwest, and Mr. Joe Reano, president of the Rotary Club of Makati North.



April 2008

Thru the donation of Ms. Maricar Banaag who responded to a plea for help during GMA 7's 'Emergency' feature on patients with MPS Hunter disease, PSOD was able to help subsidize the following:

May 2008

Consultation and lab exams (2D-echo, Optha works, BAER), of three (3) MPS Hunter Syndrome patients, as well as the Leucine monitoring subsidy of one (1) MSUD patient.

June 2008

Bone sotometry for twins with Gaucher; lab test for four (4) patients with MSUD and PKU; purchase of Salbutamol and Glocura milk for one (1) MPS Hunter Disease patient

June 2008

Bone Sotometry cost subsidy for twins with Gaucher; Lab test subsidy (NIH) for four (4) patients with MSUD and PKU; and Salbutamol and Glocura milk for one (1) MPS Hunter Disease patient

July 2008

Lab test at NIH for the four (4) patients with Galactosemia, MSUD and PKU

August 2008

Lab test at NIH for seven (7) patients with PKU and MSUD

September 2008

Lab test at NIH for eight (8) patients with PKU and MSUD

October 2008

Lab test at NIH for two (2) patients with MSUD

March 2009

Lab test at NIH for one (1) patient with MSUD

October 2008

Facilitation of C-PAP machine donation for MPS Hunter patient Jerich Duran from Tefa-Portanje (www.tefa-portanje.nl) and Dutch Neuromuscular Disease Association Netherlands thru the effort of Ms. Marize Schoneweld, a Pompe patient. PSOD sponsored the FEDEX remote pick-up fee and delivery.#



PROJECT RARE LAUNCH

The Philippine Society for Orphan Disorders, Inc. (PSOD) launched its Project Rare Program at a dinner affair last Feb. 25, 2009 at the Brasserie 21 Restaurant at the Security Bank, Ayala Ave., Makati City.

Project Rare kicked off the public awareness campaign to support the critical care of children born and afflicted with rare diseases.

The series of activities to create public awareness aims to (1) increase the registry of patients and refer them to the Institute of Human Genetics of the National Institute of Health for accurate diagnosis and access to available treatment, (2) build a network of partners and friends who can share their time, talent, and/or resources to contribute to the collective efforts to sustain a better quality of life for our member patients, and, (3) with the support of various sectors of society, build an endowment fund to sustain the life long medical treatment and therapies of patient members.

Dra. Carmencita Padilla, Chairman of PSOD, welcomed the guests comprised of current and potential donors, partners, friends, members of the medical society from Australia, Singapore and Taiwan, the patients and their families

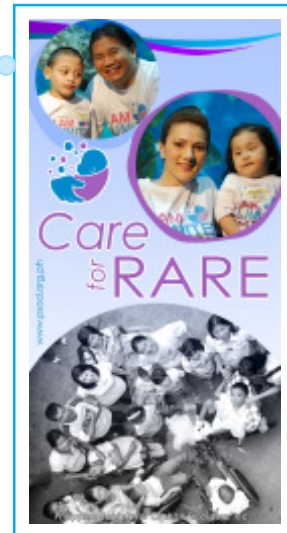
MS. Cynthia Magdaraog, President of PSOD outlined the specifics of the awareness campaign and the strategies to increase awareness, build network of partners and friends, and the start up of the Endowment Fund.

Dra. Margarita Jimenez , Medical Director for Asia Pacific of Genzyme Corporation, spoke of the various lysosomal disorders, their causes and possible treatment for patients with rare diseases under this group of disorders.

Engr. Elpidio Paras, PSOD Vice President and grandfather of Carlos Benedict Uy--a patient member afflicted with MSUD--shared with the audience their extremely personal experience that saved the life of Carlos, thru the timely

and accurate diagnosis of Dra. Padilla. Carlos at birth was just given a few hours to live. Engr Paras gave a very heart warming closing remarks for the affair.

Gracing the occasion as Guest of Honor and Keynote Speaker was Dr. Jaime Galvez Tan, former Secretary of Health who spoke of possible ways where PSOD can approach government for assistance. Dr. Galvez Tan ended his speech with "I will be your Champion (endorser)".#



Project Rare Launch Photo Gallery



Top photo: Guest of Honor Dr. Jaime Galvez Tan, former Secretary of Health, delivers his keynote address and ended his speech with the message, "I will be your Champion endorser"

Bottom photo: Carlos Benedict Uy, child with MSUD disorder pose with his family and with celebrity family endorser. (L to R) The Legaspi family, Zoren with wife Carmina Villarreal, twins Mavy and Cassy with Carlos Benedict, his mom, and grandfather Engr. Paras

Top photo, L to R: Victor Magdaraog, parent of Pompe Patient, Elpidio Paras, vice-president, Dr. Jimmy Galvez Tan, Cynthia Magdaraog, president, Dra. Carmencita Padilla, chairman of the board, Dra. Sylvia Estrada, secretary

Bottom photo, L-R: Dra. Margarita Jimenez, Medical Director, Asia Pacific Group, Genzyme Singapore orients the audience on the causes, medical manifestations, and current treatment available for rare disorders that are under the group of Lysosomal Storage Disorders

Top photo: Visitors view the information on the various rare disorders cared for by PSOD

Bottom photo: PSOD Founding Chairman Dr. Carmencita Padilla welcomes the distinguished guests of honors, partners and friends, patients and their families to the launching of Project Rare.

PROJECT RARE START-UP ACTIVITIES

2008-2009

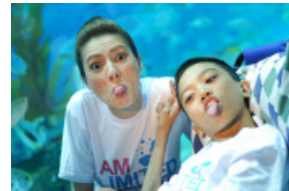
April 2008



Project Rare logo property rights application filed at the Philippine Intellectual Property Rights.

September 2008

Fun Day and Photo Shoot at the Manila Ocean Park (MOP). Tina-Mistica Santos, Marketing Manager of MOP, arranged with CDO Hotdog the sponsorship of 30 MOP entrance tickets for the patients and their families. To make the event more memorable, CDO also sponsored an interactive bubble show as entertainment for the patients and their families, the volunteer and staff of PSOD. Celebrity supporters Carmina Villarroel, Zoren Legaspi and Gabe Mercado obliged PSOD with their presence at the photoshoot to build library of photos for awareness campaign.



October 2008

GMA 7'S "UNANG HIRIT" GUESTING. Thru the efforts of Carmina Villarroel, PSOD was featured on Unang Hirit, a primetime morning TV program. PSOD President Cynthia Magdaraog and Ms. Villarroel talked about the Society and the different rare diseases. Some of the patients and their families were also present for the show.

QTV 11's "Day Off" Anniversary Presentation. PSOD was featured during Day Off's anniversary celebration. This was arranged thru the courtesy of Ms. Carmina Villarroel, PSOD's celebrity endorser, who also gifted PSOD with an awareness billboard located at the Manila Seedling Bank at the corner of Edsa and Quezon Ave as part of the show's celebration.

Imation Apollo. Taking on the concept of PROJECT RARE, Ms. Mylene Ferrer, Imation Marketing Manager, organized a Project Rare Photo Contest with exhibit caravan at SM Southmall, SM Marikina, SM Megamall Cyberzone and MOA on Nov. 14-16, 21-23, 28-30 and Dec. 3, 5-15, 2008. PSOD was given a free space for the advocacy awareness display.

PSOD Christmas Card & t-shirts (I AM RARE / I AM LIMITED EDITION). Year-round selling of PSOD all occasion cards and t-shirts to raise awareness and funds.



November 2008

42nd International Bazaar Foundation. PSOD joined the annual fund-raising exhibition organized by the Spouses of the Heads of Mission and the members of the consular corps in the Philippines, held at the PICC Forum, CCP Complex, Pasay City. PSOD was able to set up an exhibit, distribute awareness materials and sell advocacy shirts.

PinkBox. Thru the courtesy of Ms. Carmina Villarroel, a PinkBox billboard featuring Ms. Villarroel, her daughter Cassy, and the PSOD logo was prominently displayed along Guadalupe-EDSA. PinkBox carries an array of cute girls' and ladies' accessories in pink, lavender, yellow and green crafted in fashionable shapes to mesmerize the young and the young at hearts.

February 2009

Project Rare Launch. Organized to kick-off the public awareness campaign to support the critical care of children born and afflicted with rare diseases. The launch was held last February 25, 2009 at the Brasserie 21 Restaurant located at Security Bank Ayala Ave., Makati City.

CARE TO HELP?

The following patients appeal for assistance for their medication, treatment, life-saving therapies and medical equipment.

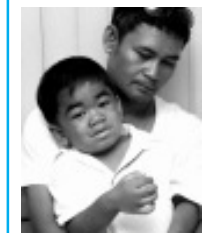
MPS HUNTER SYNDROME also known as mucopolysaccharidosis II (MPS II) is a rare inborn error of metabolism characterized by deficiency of an enzyme known as iduronate sulfatase. This enzyme breaks down specific long sugar molecules in the body called glycosaminoglycans (GAGs) that are found in many parts of the body such as the bone, joints, heart valves, etc.

Treatment: 1) Enzyme Replacement Therapy (ERT), 2) Supportive management of complications such as: pneumonia, obstructive sleep, apnea, ear infections, etc., 3) Rehabilitation therapy.

Longevity w/out meds: 15-20 years



DOMINIC RENEE DABU, 2 years old, afflicted with **MPS HUNTER SYNDROME**— looking for sponsor/s for his enzyme replacement therapy. Doctors said he has a great chance of surviving if he could be treated at an early age, unlike his brother Ivan Rey, 8 yrs old, who has advanced complications.



Dominic Renee (top)
and Carlito (bottom)

CARLITO ARBAS, 8 years old, — afflicted with **MPS HUNTER** — needs a wheelchair.

APERT SYNDROME (a craniosynostosis syndrome) is a genetic defect and falls under the broad classification of craniofacial/limb anomalies. It can be inherited from a parent who has Apert, or may be a fresh mutation. It occurs in approximately 1 per 160,000 to 200,000 live births.

Apert syndrome is primarily characterized by specific malformations of the skull, midface, hands, and feet. The skull is prematurely fused and unable to grow normally; the midface (that area of the face from the middle of the eye socket to the upper jaw) appears retruded or sunken; and the fingers and toes are fused together in varying degrees.

ALEJANDRO SAGUIN PENACO, 3 years old, afflicted with **APERT SYNDROME**. He needs help for the following;

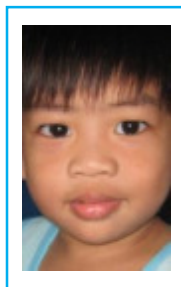
- Hand surgery,
- transportation subsidy from Mindanao to Luzon and vice versa, and
- medicines after the surgery

Alejandro is not mentally retarded but has physical abnormalities. He has club feet and hands. He had his craniofacial surgery last 2008 at the PGH and doctors said he needs another reconstructive

surgery by time he reaches 10 to 12 years old. Hand surgery is presently needed.

ADRENOLEUKODYSTROPHY (ALD), a rare, genetic disorder characterized by the breakdown or loss of the myelin sheath surrounding nerve cells in the brain and progressive dysfunction of the adrenal gland.

CHLOE DIZON, 3 years old, afflicted with ALD, need help for the following:



a. Lorenzo's oil treatment. After two months of Lorenzo's oil treatment, Chloe's blood sample will be sent to Maryland USA to check if the oil lowered his plasma VLCFA. If the trial is successful, the oil will be a maintenance for him.

b. Funds for MRI scan & anesthesiologist's fee.

PHENYLKETONURIA (PKU) is a rare inherited metabolic disease that is characterized by levels of the amino acid phenylalanine. If left untreated, excess levels of phenylalanine can cause mental retardation, seizures, movement disorders, and other serious health problems. The earlier these children are diagnosed and treated, the less risk of permanent damage.

SHAINA TAYAG, 2 years old, afflicted with PKU, needs medicine tetrahydrobiopterin (BH4).



Treatment: 1) Tetrahydrobiopterin, 2) Levodopa, 3) 5-Hydroxytryptophan, 4) Therapy, 5) Anticonvulsants. Deficiency of iduronate sulfatase leads to the accumulation of GAGs in the above organs causing their dysfunction.

MAPLE SYRUP URINE DISORDER (MSUD) is a metabolic disease that causes branched chain amino acids (leucine, isoleucine, & valine) to accumulate in the body, creating a toxic effect that can lead to brain swelling, neurological damage, and death. The disease derives its name from the sweet smell of the urine.

JEAN KATHLEEN AGUSTIN, 9 years old, afflicted with Maple Syrup Urine disorder (MSUD), needs funds for dextrose and feeding tube



Treatment: 1) MSUD formula [3 to 4 days per can], 2) plasma Amino Acid test [at least once a month], 3) Leucine level [at least once a month], and 4) Therapy

Longevity w/out meds: 7-10 days

Patients with the following rare diseases are also looking for sponsors to help defray the cost of their enzyme replacement therapy (ERT):

GAUCHER DISEASE. Twins Erika and Fatima Ligsay, 10 years old, afflicted with Gaucher disease, an inherited illness caused by a mutation in the glucocerebrosidase gene leading to the deficiency of glucocerebrosidase. Deficiency in glucocerebrosidase enzyme leads to the accumulation of glucocerebrosides in the brain, liver, spleen, skeleton, and other parts of the body leading to dysfunction of these organs.

Longevity w/o meds: Type I: 6-80 y.o.; Type II: 2 y.o.; Type III: 2-60 y.o.

POMPE DISEASE. Juan Magdaraog, 31 years old, afflicted with Pompe disease, a rare neuromuscular genetic disorder that occurs in babies, children, and adults who inherit a defective gene from their parents. There is a defect in a gene that is responsible for making an enzyme called acid alpha-glucosidase (GAA) which is either missing or in short supply. Patients suffer progressive and debilitating muscular weakness resulting in severe physical disability and dependence on ventilatory support system. The heart and lungs eventually become weak and patients finally succumb to heart and/or pulmonary failure.

Longevity w/o meds: Infantile form - 12 months; Delayed onset - 2nd and 3rd decade of life.#

CARE TO HELP?

For more information regarding the patients, how to send your donations, or how else you can be of further help, please contact:

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Seeking for Lorenzo's Oil

A story of three siblings with ALD

SIBLINGS Troy Michael, Kyle Chloe and Fiona Ysabelle were all diagnosed with Adrenoleukodystrophy (ALD).

ALD is part of a group of disorders called the leuko-dystrophies that cause damage to the myelin sheath, the fatty covering which acts as an insulator on nerve fibers in the brain.

There are several forms of ALD. Onset of the classic childhood form, which is the most severe and affects only boys, may occur between ages 4 and 10. Features of this form may include visual loss, learning disabilities, seizures, dysarthria (poorly articulated speech), dysphagia (difficulty swallowing), deafness, disturbances of gait and coordination, fatigue, intermittent vomiting, melanoderma (increased skin pigmentation), and progressive dementia.

TROY MICHAEL, who just turned six last June 18, 2008, can no longer see and is almost in a vegetative state. His story was documented on GMA 7's EMERGENCY by Arnold Clavio last OCT. 17, 2008 and it can also be watched at YOUTUBE by typing in "BATANG BIHAG NG SAKIT"(a 7-minute video clip).

KYLE CHLOE (3 y.o.) has the disease but is still asymptomatic (symptoms have not yet manifested).

There is a drug to help treat ALD. This was discovered by Augusto and Michaela Odone on their search for a cure for their son,

Lorenzo, who also suffered from ALD but died before the drug can be fully developed. The drug was named "Lorenzo's Oil" after their son (their story was made into a film in 1992 entitled *Lorenzo's Oil*, starring Nick Nolte and Susan Sarandon).

Dr. Huro Moser of Kennedy Krieger Institute studied the effects of Lorenzo's Oil on 89 boys who have the disease but does not have the symptoms yet. Out of the 89 boys, 66 did not develop MRI abnormalities, leading to the findings that all Asymptomatic ALD boy should be given Lorenzo's oil at the earliest possible time.

PSOD wasted no time in helping the family source the nearest and cheapest distributor of Lorenzo's oil, and was able to communicate with Ms. Niki Yim of Nutricia Hongkong for Kyle Chloe's initial three bottles of the oil. One box of 12 500ml bottles of Lorenzo's oil costs Euro 1170 or P77,000.

Kyle Chloe also needs a semi-annual MRI scan costing around P7,000 at the PGH plus the fee for the anesthesiology. Due to this, the Dizon family pleads for any help for Kyle Chloe to continue his oil treatment and twice a year MRI scan. They said that Kyle Chloe will be the first in our country to try Lorenzo's oil. For these reasons, the Dizon family appeals to the kindness and generosity of individuals



L-R: Troy Michael, Kyle Chole and Fiona Ysabelle.

For donations and other assistance, please contact PSOD or Michael B. Dizon at 23 A Cadena de Amor St. Bahayang Pag-asa Subd., Maysan, Valenzuela City or at tel. no. 294-4445 or mobile no. 09198243041

or organizations for help in providing and starting the Lorenzo's oil treatment for Kyle Chloe who could be the first in the country to try Lorenzo's oil. PSOD echoes this appeal. The family is willing to provide any requirement/s which may be needed to qualify for assistance.

FIONA YSABELLE, as a female, is only a carrier of the disease.

EDWIN JOHN RECEDE



EDWIN JOHN or Janjan, one of the few Filipino patients afflicted by adrenoleukodystrophy (ALD) died at the age of 15 last July 18, 2009.

A resident of Lipa, Batangas, friends and family recalls Janjan to be one of the smartest kids in his class when he was in pre-school up to grade three, the year when ALD began afflicting his body at age 8.

Adrenoleukodystrophy or ALD is a rare, genetic disorder characterized by the breakdown or loss of the myelin sheath surrounding the nerve cells in the brain and progressive dysfunction of the adrenal gland.

Treatment includes bone marrow transplantation, Lorenzo's oil, & steroid replacement. Death usually occurs within 1 to 10 years after the onset of symptoms w/out medication.

Janjan was among the children with illness featured last October 2008 on GMA7's Emergency episode entitled "Batang Bihag ng Sakit". It was his last public appearance. The video was uploaded on youtube as part of PSOD's campaign to seek assistance for Janjan's medical expenses.#

Partners in Focus: GENZYME CORPORATION

Our tomorrows are brighter because you care...

GENZYME is one of the world's leading biotechnology companies. Its more than 11,000 employees work in countries throughout the world and are united by a common goal: to make a major positive impact on the lives of people with debilitating diseases.

Since its founding in 1981, Genzyme has grown from a small start-up to a diversified enterprise with 2008 revenues of \$4.6 billion. Over the past two decades Genzyme has introduced a number of breakthrough treatments in several areas of medicine, which have provided hope to patients who previously had no viable treatment options. Genzyme products are helping patients in 100 countries.

Today, Genzyme continues to be driven by its commitment to patients. The company is working to develop new medicines, improve its existing therapies, secure approvals for its products around the world, and ensure that patients have access to these treatments. Genzyme, which has its headquarters in Cambridge, Massachusetts, was chosen in 2007 to receive the National Medal of Technology, the highest honor awarded by the President of the United States for technological innovation. Genzyme focuses on the following broad areas of medicine: Genetics Diseases, Oncology, Orthopaedics/Biosurgical Specialties, Transplant, and Genetics/Diagnostics.

Research and Development

Genzyme's research and development efforts are focused on the areas of medicine where it markets commercial products. The company also conducts research in cardiovascular disease, neurodegenerative diseases, and other areas of unmet medical need. In 2008, Genzyme invested approximately \$750 million in this work, or about 16 percent of its revenues.

Genzyme continues to build its pipeline through both internal research and licensing and acquisitions, focusing on product candidates with the potential to change the standard of care for serious diseases. The company's broad pipeline features seven major late-stage programs, including: alemtuzumab for multiple sclerosis; Clolar for adult acute myeloid leukemia; and mipomersen for familial hypercholesterolemia and other high risk, high cholesterol patients.

Corporate Responsibility

Corporate responsibility is a priority at Genzyme. The company's commitment to patients extends beyond the development of new treatments and the services needed to deliver them. Genzyme has a strong presence in developing countries, where it provides free medicine to patients and helps to build sustainable health care systems. The company in 2006 launched an initiative to support the development of treatments for neglected diseases that affect hundred of millions of people in the developing world. Genzyme's headquarters is one of the most environmentally responsible office buildings in the world, and the company is a leader in waste-reduction and recycling efforts.

In addition, Genzyme supports science education and health initiatives in the communities in which it operates. For these and other efforts, Genzyme has been consistently included in the Dow Jones Sustainability World Index, which consists of companies that excel in economic, environmental and social performance. For the past three years, Genzyme was named one of the "Global 100 Most Sustainable Corporations in the World" by Innovest Strategic Value Advisors. BusinessWeek has ranked Genzyme as one of the top corporate givers, and the company has also been recognized by the U.S. Environmental Protection Agency, the American Association for the Advancement of Science, and many other organizations. (For more info, visit www.genzyme.com)

Our passion is to make a difference for patients, wherever the need exists.



Genzyme Corporation has enzyme replacement therapy (ERT) for patients with Fabry Disease, Gaucher Disease, MPS I, MPS II and Pompe Disease in the Philippines.



Genzyme Corporation, through the coordination of Director for Southeast Asia Patrick Granjard and Genzyme Philippines Country Manager Jose Santiago A. dela Cruz, Jr., is PSOD's major partner in providing treatment to patients with Gaucher and Pompe disease. Photo: Fatima and Erika, twins afflicted with Gaucher disease, recipients of Cerezyme (enzyme replacement therapy), pose with (L-R) Dra. Margarita Jimenez, Genzyme Medical Director, Asia Pacific Group; Dr. Patrick Granjard; Dra. Lynn Silao, Board of Director of PSOD; Vilma Ligsay, mother of the twins; Jose Santiago dela Cruz; and Danilo Ligsay, father of the twins, during the Project Rare Launch, February 25, 2009.

INSTITUTE OF HUMAN GENETICS



The Institute of Human Genetics provides services through its Genetics Clinics, specialized laboratories, and research unit.

The component units include:

- ▶ Cytogenetics Laboratory
- ▶ Molecular Genetics Laboratory
- ▶ Genetics Clinics at PGH
- ▶ Newborn Screening Laboratory
- ▶ Biochemical Genetics Laboratory
- ▶ Genetics Research Unit

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