Pre-owned Electronics Used to Raise Money for Orphan Drug Development

Collecting pre-owned electronics to fundraise for March of Dimes in order to raise money for the research and development of orphan drugs to help manage symptoms of orphan diseases

Tag words: Pre-owned electronics for March of Dimes, Raising money for orphan drug development

Authors: Valeriya Posternak, Matthew Knox, Artur Ivchenko with Julie M. Fagan, Ph.D.

Summary

In the last few years, the number of orphan drugs approved by the FDA has decreased due to the decrease in funding for the research and development of these drugs. Many of these orphan diseases, diseases that affect a small portion of the population, begin at birth or at a very young age; and in order to raise funds for the research and development of orphan drugs to treat these diseases, a fundraiser has been set up in association with March of Dimes. A bin was set up at Gamestop, a video game and electronics store, in order to collect used electronics and cell phones, which were then sent out to March of Dimes.
Issue: Orphan Drug Development

Number of People Suffering from Diseases
As of 2009, the total number of people residing in the United States is 308,075,522 (U.S. Census Bureau, 2009); and, in the United States alone, there are millions of people suffering from diseases, both curable and incurable. For example, heart disease is the number one killer in the country, and the “number of noninstitutionalized adults with diagnosed heart disease is 26.6 million (12%)” (Centers for Disease Control and Prevention, 2009). Approximately, 11,028,000 people (3.6%) in the United States are afflicted with invasive cancers (Centers for Disease Control and Prevention, 2009). Furthermore, an estimated 5 million Americans (1.6% of the US population) have Alzheimer’s disease. The number of people suffering from Alzheimer’s has doubled since 1980, and is expected to be as high as 13.4 million by 2050 (Centers for Disease Control and Prevention, 2009). These diseases, as compared to others in the United States, are quite common and researchers studying possible cures for these diseases are provided with a great deal of funding from the government, private organizations, and investors. The money provided for the research and development of various drugs, such as, ACE inhibitors, beta blockers, Amiodarone, and Digoxin for heart disease, and such as Aricept oral, Cognex oral, and Exelon oral for Alzheimer’s disease, is causing a rapid increase in the amount of medicines put on market. With an increase in the drugs on the market for a certain disease, the chance of those drugs aiding a larger portion of the population is increased as well. Drug on the market has the potential to cater to the specific needs of an individual. This potential, however, is not seen with diseases such as Fabry disease, which is a “inherited lysosomal storage disorder caused by the partial or complete deficiency of the lysosomal enzyme alpha galactosidase A (alpha-Gal A), resulting in excess cellular glycosphingolipid deposition” (Pierre-Louis B et al, 2009:1). Fabry disease is an orphan disease. In the United States, orphan diseases are classified as “diseases that afflict fewer than 7.5 out of 10,000 people, or roughly 200,000 Americans, and ultra-orphan diseases are generally defined as those that afflict fewer than 10,000 people globally” (Finegold et al, 2005:57). Fabry disease is present in 40,000 males globally. It is estimated that there are approximately 3,800 males in the United States that suffer from Fabry disease, and 7,600 females are estimated to carry the Fabry gene. Altogether, the number of affected individuals is approximately 10,000, 0.003% of the US population. Genzyme has recently made progress in the medical field with the development of Fabrazyme as a treatment for Fabry disease. This is just one of many orphan diseases. Disease presents yet another example, and occurs in “an estimated 2 to 4 of every 100,000 live births” (National Institute of Neurological Disorders and Stroke, 2009) 9,424 making the total population of people afflicted with the disease, inherited disorder, Batten disease is a fatal, in the United States (100,000 out of 3 average) Unlike Fabry, it is the age of starting between the ages of that affects the nervous systems, as are many other orphan diseases, this disease is currently untreatable, disease.

It is evident that a single orphan disease affects a very population of individuals when compared with the total population of the United States. However, a total of approximately 6,000 orphan diseases affect roughly 25 million individuals across the country. This number has a collective significant impact. A comparison of the number of individuals afflicted with common diseases as compared to those suffering from orphan diseases can be seen in Figure 1: Number of People Suffering From Diseases.
Rare diseases “are life threatening or chronically debilitating diseases with a low prevalence and Rare diseases “the others are rare cancers; Most of them are genetic diseases, a high level of complexity, toxic and infectious diseases among other, congenital malformations, autoimmune diseases, extremely diverse Rare diseases are.” (2008:2, Europe’s Challenge: Rare Diseases) categories” but they do in fact have some common characteristics aiding in their classification as such “diseases are severe to very severe, chronic, often degenerative and life threatening, The onset of the disease occurs in childhood for 50% of rare diseases” (Diseases: Understanding this Public Health Priority, 2005:5) making the quality of life for rare, The diseases are often disabling. The disease patients almost unbearable due to their consequent lack of independence, hological burdens are extremely painful for those suffering from rare psychosocial and psychosocial and psychological burdens are extremely painful for those suffering from rare diseases and close loved ones, disease and hope for cures and an absence of practical support for everyday life from the people and companies around them.

In the last two decades, two acts were passed in the hopes of increasing the amount of company participation in the research and development of drugs for orphan diseases. These two acts, the Orphan Drug Act of 1983 and the Rare Disease Act of 2002, provided incentives and the motivation necessary for biotech and pharmaceutical companies to join the fight for the cure and treatment of orphan diseases. Prior to the passing of the Orphan Drug Act, only 10 orphan drugs were approved by the FDA, and since the passing of the act in 1983, the number of drug approvals for orphan diseases has risen to 160 between the years of 1984 to 2005.

Influences on Research and Development: Decrease in Orphan Drugs

Thus, the passing of these acts, .ABRAZYME® AGALSIDASE BETA (IS INTENDED TO REPLACE THE MISSING ENZYME. .ABRAZYME IS PRODUCED USING RECOMBINANT GENETIC TECHNOLOGY. THE RESULT IS AN ENZYME THAT IS NEARLY IDENTICAL TO THE ALPHA-GAL ENZYME PRODUCED BY THE BODY.)
CURE FOR A POTENTIAL BUT THE TREATMENT IS, TUGS PUT ON THE MARKETO THOSE OF OTHER DR
AVE BEEN MADE VEN THOUGH STRIDES HỆ. ABRY DISEASE F THOSE SUFFERING FROM
T TO SEE ANY SORT OF ATTEN DISEASE HAS YEB, THE TWO ACTS SINCE THE PASSING OF
ETWEEN THE B. 2009 OR TREATMENT AS OF AND THERE IS NO DRUG, MEDICAL BREAKTHROUGH
VAL OF ORPHAN DRUGS NCREASE IN THE APPRO THERE WAS A STEADY 1, 2000 AND 1990 YEARS OF
FDA EDUGS APPROVED BY TH THE NUMBER OF ORPHAN, DEBUT IN THE LAST DECA, FDA BY THE
APPROVALS FOR A U MULATIVE C: FIGURE F WHICH CAN BE SEEN IN, HAS DECREASED
2008-1990 EDICINESM ATED TO RUG APPROVAL IS ESTINE ORPHAN DÒ
ARE ESTIMATED TO 1983 D SINCE ORPHAN DRUGS APPROVE 216 HE T. DEATHS 211 PREVENT UP TO
ITH A W. (2003, IRZ A M) ”EASES DEATHS FROM RARE DIS 108,000 YHAVE PREVENTED NEARL
IN FDA MARKET BY THE EEING APPROVED FOR THNT OF ORPHAN DRUGS BDECREASE IN THE AMOU
YES HAVE BEEN LOSTA LARGE NUMBER OF LI, YEARS 7 OR 6 THE LAST

Figure 2: Cumulative Approvals for Medicines 1990-2008

The approval of any type of drug is based upon the ability of the research team to prove to the
FDA that the drug is effective and safe. This is done through clinical trials, and without the
funding to start or complete clinical trials, researchers of orphan drugs are unable to show the
FDA that the drug is effective and safe. A small patient population also hinders the process due
to the fact that the drug is tested on a smaller population, and thus, not as many tests can be
performed to assess the effectiveness and safety of the product. Companies are also unwilling to
initiate the research on orphan drugs due to the smaller potential profit gained when a small
population is involved.
Decrease in Funding for Research and Development of Orphan Drugs

It is clear that there has been a decrease in the amount of funding being provided for the research and development of orphan drugs in the last decade or so, while funding for other areas of research has increased. As per the National Institute of Health Research Portfolio, cancer research was allotted $5,639,000,000 in 2005, and that area of research is expected to receive $6,016,000,000 in 2010 (National Institutes of Health, 2009). Money given to researchers studying cancer has increased, while the money allotted to those doing research of orphan diseases and drugs has decreased. In 2005, $1,228,000,000 was allotted to orphan drug research, and $678,000,000 is expected to be allotted to orphan drug research in 2010 (National Institutes of Health, 2009), which is almost half as much as was previously provided. This data is based upon grants, contracts, and other funding used across the National Institutes of Health (NIH).

By dividing the above amount of $678,000,000 by the number of known rare diseases (NIH) previously mentioned, there are a total of 6,000 known rare diseases. The estimated amount of funds allotted for the research and development of an orphan drug for each orphan disease is $113,000. This data is based upon grants, contracts, and other funding used across the National Institutes of Health (NIH).

It is also visible that as per the Office of Orphan Products Development, the amount of money provided for the research and development of an orphan drug for the disease falling under the research portfolio is $113,000. It is also visible that the research portfolio was separate from that of orphan diseases. The amount of money provided for the research and development of an orphan drug for the disease falling under the research portfolio is $113,000. The amount of money provided for the research and development of an orphan drug for the disease falling under the research portfolio is $113,000.

The $14.392 million appropriated in FY 2005 for research will be used to fund 11 to 14 new grants and maintain approximately 60 ongoing grant-funded clinical study projects” (Office of Orphan Products Development, 2006). Accompanied by a decrease in the amount of funding being provided, the number of grants awarded by the department has been decreasing year after year as a result of a continued increase in the cost of clinical trials. Even though the number of grants awarded has declined, the number of applications has steadily increased, which means that researchers are desperately in need of funding. With the cost of developing a new drug, orphan or not, increasing from $100,000,000 in 1979 to $1,300,000,000 in 2005, the approximate amount of $113,000 allotted to each orphan disease does not nearly cover the cost of development; this is especially true if one were to take into account the possibility of needing to develop more than one drug. The costs associated with the development of new drugs can be seen in Figure 3: Cost to Develop New Drug.
Small patient population
Another reason for the decrease in the amount of orphan drug approvals is the result of companies’ decreased motivation to conduct the research and development necessary due to the small patient populations usually associated with orphan diseases. “Rare diseases are generally neglected by pharmaceutical companies due to their relatively small patient populations. Biotech companies, on the other hand, do produce therapies to treat rare and orphan diseases, but patient treatment costs are often prohibitively expensive” (Agres, 2006:6). Even with all of the incentives introduced by the Orphan Drug Act, including the fact that a sponsor may claim 50% of clinical trial costs as a credit against taxes owed and product exclusivity for seven years (no creation of generics), the amount of money made through the development of orphan drugs is substantially lower than money made by developing drugs for more common diseases. “Drugs for rare diseases—so-called orphan drugs—can be difficult to research, develop, and market. Proof of drug safety and efficacy in small populations must be established, but doing so is a complex process. In addition, the cost of developing a drug can greatly influence priorities when the target population is relatively small” (Berkowitz, 2009).

A 30-day supply of Fabrazyme, the only drug available for people suffering from Fabry disease, has a cost of $4,542.30. If this is multiplied by the total number of people suffering from Fabry disease, a population of approximately 10,000, the total profit is $45,423,000. On the other hand, Aricept, which is a drug that treats Alzheimer’s patients, has a cost of $222.61 for a 30-day supply. If $222.61 is multiplied by the total number of people suffering from Alzheimer’s disease, a population of approximately 5 million, the total profit is $1,108,050,000. This is a significant difference in profit: Aricept makes a profit of almost 25 times as much as Fabrazyme. Companies, like Genzyme (produces Fabrazyme), are therefore forced to charge more for their orphan drugs, but not even the escalated cost of the drug matches the profit made by companies producing drugs for more common diseases like Alzheimer’s. The profits made from Fabrazyme take longer to cover the cost of the drug’s development as compared to the amount of time it takes for the profits of a more common drug, like Aricept, to cover the cost of its development. The cost of clinical trials for drugs, excluding orphan drugs, is between $100 M and $800 M. For orphan clinical trials, sponsors have the ability to claim 50% of clinical trial costs as a credit toward taxes, meaning orphan clinical trials are between $50 M to $400 M. For a drug like Aricept, at a cost of $221.61 for a 30-day supply, it would take 2.7 to 21.7 days to cover the cost of clinical trials ($100 M to $800 M), while a drug like Fabrazyme, at a cost of $4,542.30 for a 30-day supply, would take 33 to 264 days to cover the cost of clinical trials ($50 M to $400 M). It takes almost 12 times longer for Fabrazyme to cover the cost of clinical trials than it takes for Aricept to cover the cost of clinical trials that are twice as much.

Service Project:
Orphan diseases affect many infants at birth or not too long after birth, such as Batten’s disease and Fabry disease. Many specific organizations are involved in fundraising for orphan diseases in order to increase the research and development of orphan drugs. One such organization is March of Dimes, United States health charity whose mission is to improve the health of babies by preventing birth defects, premature birth, and infant mortality. They take donations in various ways, including money, cars, stock, change, and used electronics and cell phones. What we have done is collaborate with March of Dimes through their activity of collecting old electronics and used cell phones. Our particular activity is to have a table at the Douglass Campus with a bin to collect said electronics on Thursday, April 29th between 1PM and 3PM.
We will also be discussing what March of Dimes accomplishes and how valuable it can be to people who think they have no way in which to help themselves. We will also have bins available at the two entrances to the Douglass Campus Center during Rutgers Day between 10AM and 4PM on Saturday, April 24th. In addition to this act, we have coordinated with Gamestop in Middletown, NJ to try and supplement our activities in the campus center. A bin and a sign have been set up at the register in Gamestop in order for customers to be fully exposed to the service project upon purchasing products. An editorial was submitted to the Daily Targum at Rutgers University and another one was submitted to The Star Ledger of NJ to promote the collection of electronics at the Douglass Campus Center on April 24th (Rutgers Day) and April 29th. Another editorial was submitted to the Asbury Park Press, a local newspaper, to promote the collection of used electronics at Gamestop in Middletown, NJ. A page on Facebook has also been created to promote the fundraisers at all of the above locations as well as the research and development of orphan drugs. Upon collecting the electronics we will ship them back to the March of Dimes HQ in White Plains, NY, where they will use the items to raise money for research.

References


Batten Disease Fact Sheet. (2009, October 23). Retrieved March 6, 2010 from National Institute of Neurological Disorders and Stroke:

http://www.ninds.nih.gov/disorders/batten/detail_batten.htm


Katzung BG: Basic & Clinical Pharmacology, 11e:


Centers for Disease Control and Prevention. (2009, December 1). Retrieved February 28, 2010 from National Center for Health Marketing:

http://www.cdc.gov/

Estimates of Funding for Various Research, Condition, and Disease Categories (RCDC). (2009, May). Retrieved on March 7, 2010 from National Institutes of Health:

http://report.nih.gov/rcdc/categories/


http://www.fabrazyme.com/patient/product/fz_us_pt_pd_about.asp


Editorials

Donate Your Used Electronics and Cell Phones to Help Support and Raise Money for Orphan Drug Research and Development

Valeriya Posternak

Orphan drug research and the number of orphan drugs approved by the FDA have increased in the last 25 years due to incentives put in place by the Orphan Drug Act of 1983. These incentives influenced biotech companies to develop orphan drugs for orphan diseases when, in the past, nobody else would. An orphan disease is one that affects 7.5 out of 10,000 people, or roughly 200,000 Americans, and ultra-orphan diseases are generally defined as those that afflict fewer than 10,000 people globally. Orphan diseases affect few people, but the symptoms are devastating and the lives of those afflicted are torturous. This is the reason for the demand for orphan drug research and development by the public.

Orphan drug research and development has increased since the 1980s, however, the number of orphan drug approvals per year has decreased in the last few years. This decrease in approvals by the FDA is due to the fact that companies are no longer capable of completing clinical trials. Overall, the decrease in funding for orphan drug research and development has caused this decrease in approvals.

In order to help support the cause for raising awareness as well as funding for orphan drug development, a fundraiser was planned in association with March of Dimes. March of Dimes is a United States health charity whose mission is to improve the health of babies by preventing birth defects, premature birth, and infant mortality.

Many orphan diseases affect infants at birth or not too long after birth, and March of Dimes raises funds for research of cures for various diseases and defects that affect babies. In support of babies, March of Dimes collects used electronics and used cell phones. For this reason, a bin was set up at Gamestop in Middletown, NJ for the purpose of collecting these items. Donations have been made at Gamestop, but more are needed.

We will be in the Douglass Campus Center (DCC) on the corner of George St. and Nichol Ave. between 1PM and 3PM on April 29th collecting used electronics and cell phones. We will also be collecting these items on Rutgers Day between 10AM and 4PM at the DCC at the two entrances. We’ll then send all of these donations out to the March of Dimes headquarters located in White Plains, NY where they will use them as funding for the research and development of drugs for infant defects, including orphan diseases. If you have any questions, please feel free to contact me at 848.466.7215.

Valeriya is a microbial biotechnology major at Rutgers University.

Help Support Orphan Drug Research and Development by Donating Your Used Electronics

Matthew Knox

An orphan disease is one that affects 7.5 out of 10,000 people, or roughly 200,000 Americans, and ultra-orphan diseases are generally defined as those that afflict fewer than 10,000 people
globally. Orphan diseases affect few people, but the symptoms are devastating and the lives of those afflicted are torturous. The number of orphan drugs approved by the FDA has been small due to two main reasons: small patient population & decrease in funding for research and development of orphan drugs.

A small patient population has an immense effect on productivity and profit. Companies that do develop orphan drugs will charge an insane amount of money compared to companies that make drugs for more common diseases. This is done to increase profit for companies that develop these orphan drugs when the effected population is small. However, no matter how you look at it, the amount of money made through the development of orphan drugs is substantially lower than money made by developing drugs for more common diseases.

Orphan drug research and the number of orphan drugs approved by the FDA have increased in the last 25 years due to incentives put in place by the Orphan Drug Act of 1983. These incentives influenced biotech companies to develop orphan drugs for orphan diseases. Orphan drug research and development has increased since the 1980s, however, the number of orphan drug approvals per year has decreased in the last few years. The main cause for this decrease in orphan drug approvals is the decrease in funding for orphan drug research and development.

In order to help support the cause for raising awareness as well as funding for orphan drug development, a fundraiser was planned in association with March of Dimes. March of Dimes is a United States health charity whose mission is to improve the health of babies by preventing birth defects, premature birth, and infant mortality. Many orphan diseases affect infants at birth or not too long after birth, and March of Dimes raises funds for research of cures for various diseases and defects that affect babies. In support of babies, March of Dimes collects used electronics and used cell phones. We have set a bin up at Gamestop in Middletown, NJ for the purpose of collecting these items. Donations will be taken at all time during Gamestop hours of operation 7 days a week. Donation will be sent out to the March of Dimes headquarters located in White Plains, NY upon collection. Feel free to stop by and make your donation. Your donation will make the difference. If you have any questions, please feel free to contact me at 908.601.9314.

Matthew is both majoring in biological sciences at Rutgers University.

Come Help Support Orphan Drug Research and Development on Rutgers Day at Rutgers University

Artur Ivchenko

Rutgers Day at is a celebration of our great university and our great state of New Jersey. One of the main purposes of Rutgers Day is to teach other about the great state of New Jersey as well as all of the accomplishments and research being done at Rutgers University. Research is a large part of education at the university.

In the last 25 years, orphan diseases have been in need of the research and development of drugs to ease those suffering. An orphan disease is one that affects 7.5 out of 10,000 people, or roughly 200,000 Americans, and ultra-orphan diseases are generally defined as those that afflict fewer than 10,000 people globally. Orphan diseases affect few people, however, the symptoms
are unlike any other common disease. Orphan drug research and the number of orphan drugs approved by the FDA have increased in the last 25 years due to incentives put in place by the Orphan Drug Act of 1983. These incentives have influenced companies to develop orphan drugs for orphan diseases.

Orphan drug research and development has increased since the 1980s, however, the number of orphan drug approvals per year has decreased in the last few years. This decrease in approvals by the FDA is due to two reasons: small patient population and the decrease in funding for research and development of orphan drugs. A small patient population decreases potential profits made while decrease in funding prevents the completion of clinical trials.

In order to help support the cause for raising awareness as well as funding for orphan drug development, a fundraiser was planned in association with March of Dimes. March of Dimes is a United States health charity whose mission is to improve the health of babies by preventing birth defects, premature birth, and infant mortality.

Many orphan diseases affect infants at birth or not too long after birth, and March of Dimes raises funds for research of cures for various diseases and defects that affect babies. In support of babies, March of Dimes collects used electronics and used cell phones. For this reason, a bin was set up at Gamestop in Middletown, NJ for the purpose of collecting these items. Donations have been made at Gamestop, but more are needed.

Bins will be set up at the two entrances to the Douglass Campus Center (DCC) located on the corner of George St. and Nichol Ave. between 10AM and 4PM on Rutgers Day on April 24th for the purpose of collecting used electronics and cell phones. We will also be at the DCC on Thursday, April 29 between 1PM and 3PM collecting these items. All donations will be sent out to the March of Dimes headquarters located in White Plains, NY where they will use them as funding for research, including orphan drug research. If you have any questions, feel free to contact me at 848.459.0395.

Aruitr is a biological sciences major at Rutgers University.