



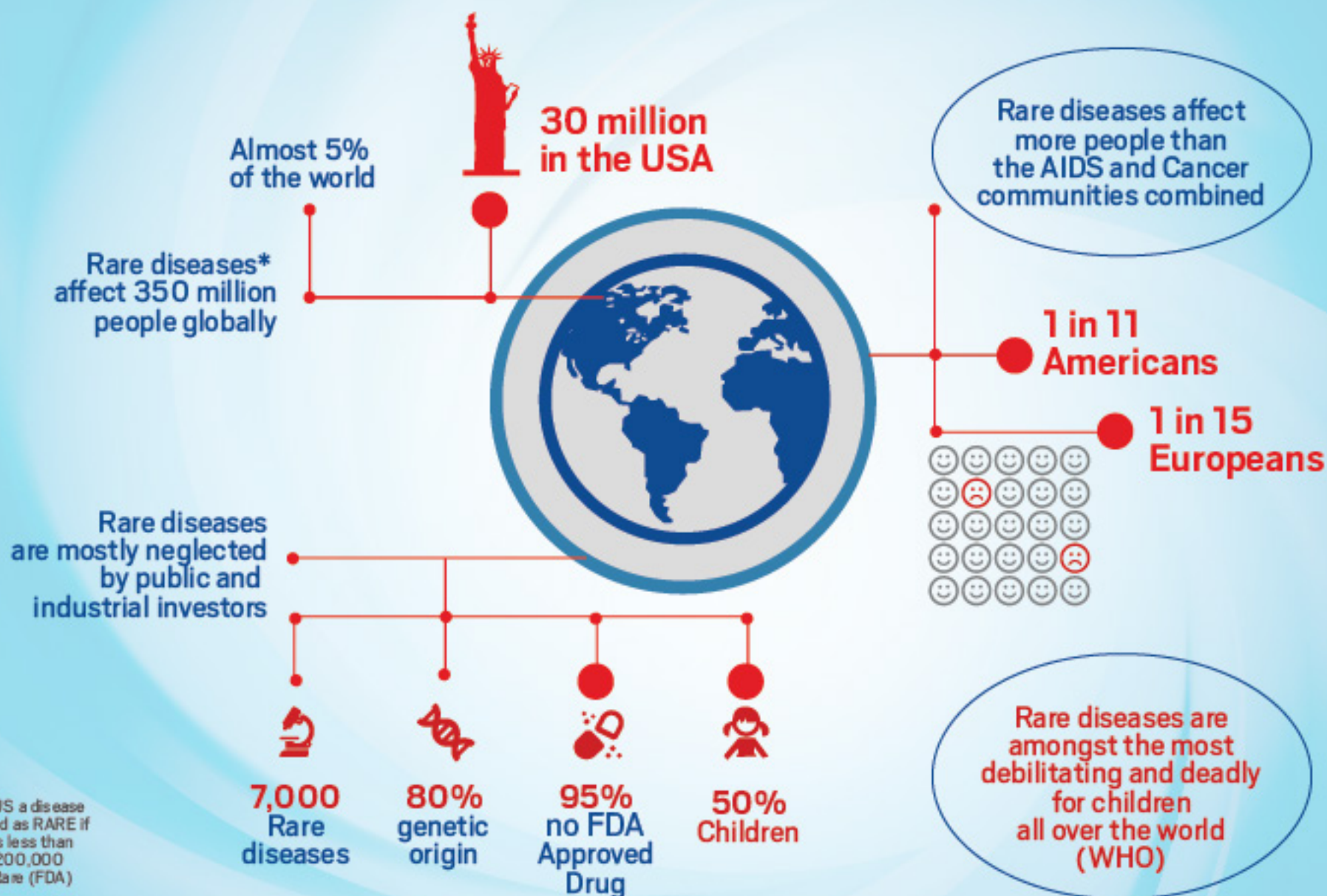


# RARE DISEASES

A SILENT EMERGENCY



# An Emergency



\*In the US a disease is defined as RARE if it affects less than 7,000 200,000 people Rare (FDA)

# To Neglect Rare is to Neglect Children



**10 CHILDREN**

in the world  
are born with  
a genetic disease

**EVERY MINUTE**



**70% OF CHILDREN**

affected by a genetic  
disease show symptoms

**BY AGE 3**



Rare diseases are  
responsible  
for **35% OF DEATHS**

**IN THE FIRST  
YEAR OF LIFE**



  
**5**

More than  
**1 in 3 CHILDREN**  
affected by a rare  
genetic disease

today only make it to  
**AGE 5**



# An Emergency for Those Considered too Rare



 [www.daybreakfoundation.org](http://www.daybreakfoundation.org)

Rare diseases are  
**neglected**

by public investors  
and pharmaceutical companies

Millions of people  
don't even have  
the hope for a cure

This is why  
charitable giving  
is irreplaceable



SHARON  
with her dad





# EVERY PERSON

COUNTS



# Every Person Counts



 [www.daybreakfoundation.org](http://www.daybreakfoundation.org)

Our vision is a world where

**all genetic diseases  
are cured**

and no patient or illness  
is neglected

**We believe  
no child is too rare  
to deserve a cure**



UMA

# Our mission



 [www.daybreakfoundation.org](http://www.daybreakfoundation.org)

Daybreak  
Children's Rare Disease Fund  
is a not-for-profit organization  
whose mission is to support only

**the very  
best research**

aimed at the cure of

**rare genetic  
diseases**

**GIOVANNI**

Omaha, NE

affected by MLD, was treated  
with gene therapy supported by Daybreak  
and now is healthy





# People



[www.daybreakfoundation.org](http://www.daybreakfoundation.org)

## BOARD OF DIRECTORS

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# Our Events

The Gala for the 60th Anniversary of Ferrari North America  
hosted a thrilling auction. A beautiful

## **Ferrari 458 Speciale A**

was adjudicated for

**\$ 900,000**

to benefit Daybreak







# FUNDING

ONLY THE BEST RESEARCH

# Who We Fund



 [www.daybreakfoundation.org](http://www.daybreakfoundation.org)

Daybreak chose as its primary beneficiary one of the world's leading institutions in gene therapy and research on rare genetic diseases:  
**Italy's Telethon Foundation**  
and its two cutting-edge centers

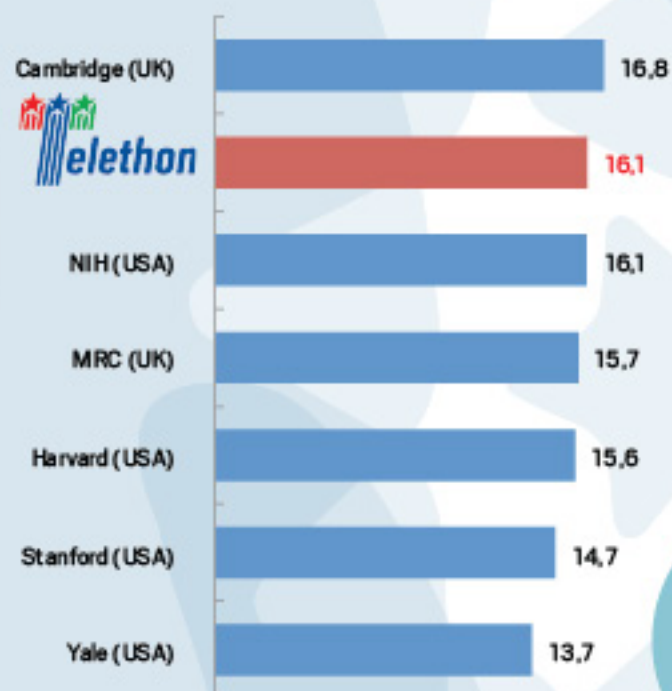
GINEVRA  
with mom and dad





# We Chose a Track Record of Excellence

## TELETHON COMPARED TO INTERNATIONAL INSTITUTIONS



Citation Index  
Average number  
of citations/paper  
2009-14  
(Source: Thomson Reuters)

## THE METHOD

We support only top,  
internationally competitive  
projects through a highly  
selective peer review conducted  
by a prestigious international  
Commission

ONLY THE BEST BASIC  
AND CLINICAL  
RESEARCH

**\$573M**  
Invested

**20+**  
Rare Diseases  
in clinical or  
pre-clinical  
Stages  
(15)

**449**  
Rare Diseases  
Studied

**\$82.1M**  
Revenue 2014

48.7 gifts  
28 grants  
5 financial  
0.5 other

**77%**  
Spent  
in Programs  
(14)

**2,532**  
Research  
Grants


**9,836**  
Scientific  
Publications  
(1990-2014)

**1,547**  
Research  
Teams

**378**  
Telethon  
Staff

# We Chose Changing Lives Worldwide



 [www.daybreakfoundation.org](http://www.daybreakfoundation.org)



Daybreak  
funded  
Clinical Trials

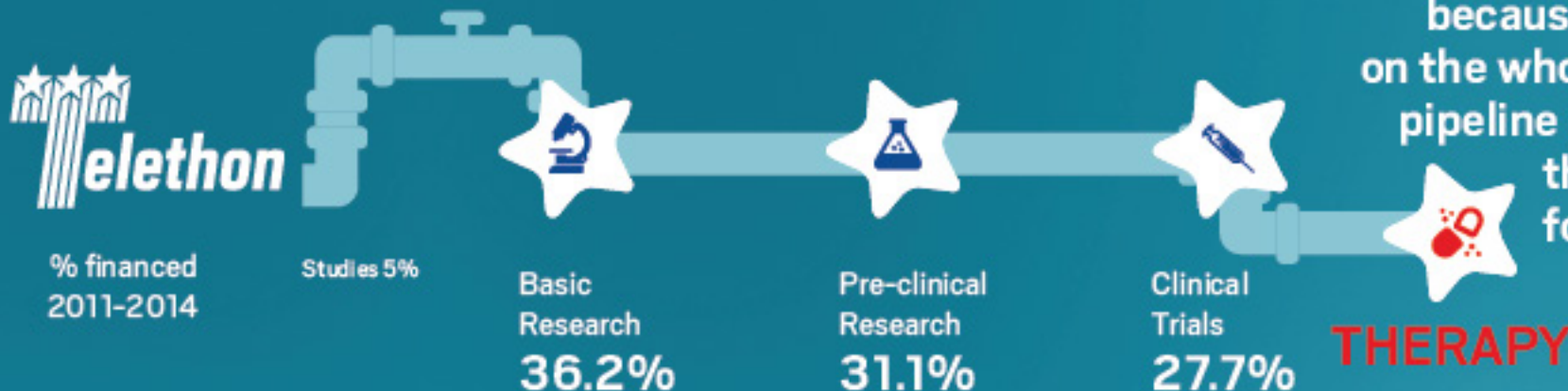
100%  
Survival Rate

Ellen Pompeo (Grey's Anatomy)  
Watch her appeal to support Daybreak  
[www.daybreakfoundation.org](http://www.daybreakfoundation.org)





# We Chose to Bring Research from Labs to the Life of People



We choose Italy's Telethon because they work on the whole research pipeline maximizing the potential for therapies

## Early Pre-Clinical Development

Chronic granulomatous disorder\*  
Globoid leukodystrophy\*  
Mucopolisaccharidosys type IIIa  
Leber congenital amaurosis  
Hemophilia  
Stargardt disease  
Alpha 1-antitrypsin deficiency  
IPEX syndrome  
Usher syndrome  
Pyruvate Dehydrogenase complex deficiency  
Crigler-Najjar syndrome  
Pompe disease  
Wilson disease  
Retinitis pigmentosa

## Advanced Pre-Clinical Development

Mucopolysaccharosis type I\*  
Mucopolysaccharosis type IV

## Clinical Trial

ADA-SCID\*  
Beta thalassemia\*  
Metachromatic leucodystrophy\*  
Wiskott-Aldrich syndrome\*

\*industrial alliances



# We Listened to These Voices



**Giovanni and Cecilia** (Omaha, NE) were treated with TIGET's gene therapy. Diagnosing Liviana, their late sister also affected by MLD, saved their lives.



**Sebastian** (San Francisco, CA) was treated at TIGET with gene therapy and has now been ADA-SCID free for 6 years.



**Eli and Ella** (Philadelphia, PA) are siblings both affected by MLD. Their recent treatment with gene therapy at TIGET is giving great results.



Nicoletta is a donor because today, thanks to TIGET's gene therapy, her son **Samuel**, affected by Wiskott-Aldrich Syndrome, is a healthy child.



**Francesco** was just like his superheroes: SpiderCiccio. He fought Pompe Disease. One day, thanks to research, children like him will be able to win that fight



# About Us and Our Beneficiary



**THOMAS RANDO**  
Stanford University

Being part of the  
Scientific Committee  
has been very special  
to me



**MICHAEL CAPLAN**  
Yale University

By any metric, the  
research funded by  
Daybreak is among  
the very best in the  
world

**SUSANNA AGNELLI**  
Founder

Telethon will continue  
to exist until the word  
CURE will be written  
next to the name of all  
genetic diseases



**LUCA di  
MONTEZEMOLO**  
President

We value the  
therapeutic potential  
of the research we  
fund





**WHY GIVE**  
TO DAYBREAK



# Fund Only the Best Research



 [www.daybreakfoundation.org](http://www.daybreakfoundation.org)

The research  
we fund saves children  
**from the US  
and all over  
the world**

The research we fund is  
**close to therapy**  
for over **20** rare diseases

The International Rare Disease  
Research Consortium's goal is to find  
200 therapies by 2020

## **SEBASTIAN**

(San Francisco, CA) Affected by SCID,  
was treated with gene therapy  
we fund and is now healthy



# Give Where It Counts



 [www.daybreakfoundation.org](http://www.daybreakfoundation.org)

## Efficiency

in the allocation of financial and human resources is crucial for research on rare diseases so that the centers that have specialized experience and are closer to therapy can reach results

**as fast as possible**

Daybreak makes sure this happens

**Patients can't wait**





# Maximize Gift Impact



 [www.daybreakfoundation.org](http://www.daybreakfoundation.org)

In the past 20 years, investment in research has doubled (117 billions \$ in USA) and charitable giving accounts for 4%

## **It's different for rare diseases.**

Public investors have budgetary constraints and social priorities. Pharma invest in highly profitable areas such as biotechnologies and devices

**For rare diseases  
individual gifts  
make the difference**



MAYA



# Not Only Rare



Research on rare diseases  
has a growing  
**impact** on  
more common diseases

- Hypertension and kidneys
- Hereditary cardiovascular pathologies
- Oncogenesis
- Innovative health policies



GABRIELE



**Make Your Donation Today**



 [www.daybreakfoundation.org](http://www.daybreakfoundation.org)

**BETHERE  
FOR RARE**

[www.daybreakfoundation.org](http://www.daybreakfoundation.org)









info@daybreakfoundation.org  
www.daybreakfoundation.org

