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Συμμετοχή Ασθενών στην Κλινική Έρευνα: «καλές πρακτικές και προκλήσεις για τη φαρμακοβιομηχανία»

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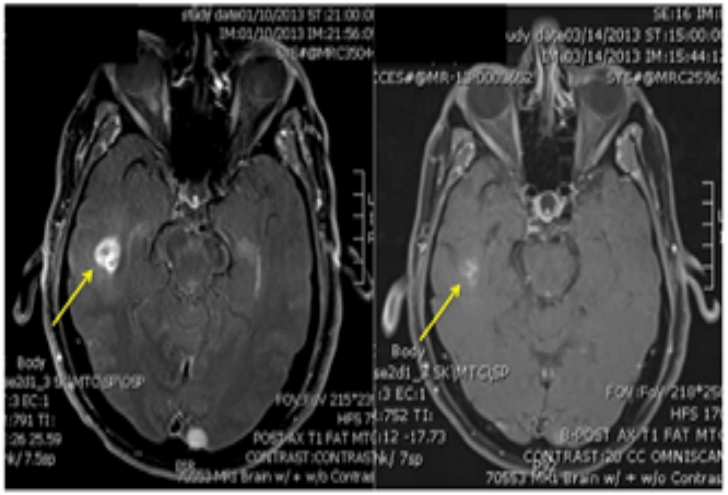
Κοινός Στόχος στην Έρευνα και Ανάπτυξη

Οι Ασθενείς Χρειάζονται Αποτελεσματικές Θεραπείες

Η Φαρμακευτική Χρειάζεται να Αναπτύξει Αποτελεσματικές Θεραπείες

Από την Αποτελεσματικότητα Στο Κλινικό Όφελος για τον Ασθενή

Μπορούμε να καταλάβουμε από την MRI πως αισθάνεται/λειτουργεί ο ασθενής?



January 10, 2013 March 14, 2013

Οι παρακάτω ερωτήσεις είναι βοηθούν να αξιολογήσουμε την κατάσταση του ασθενή?

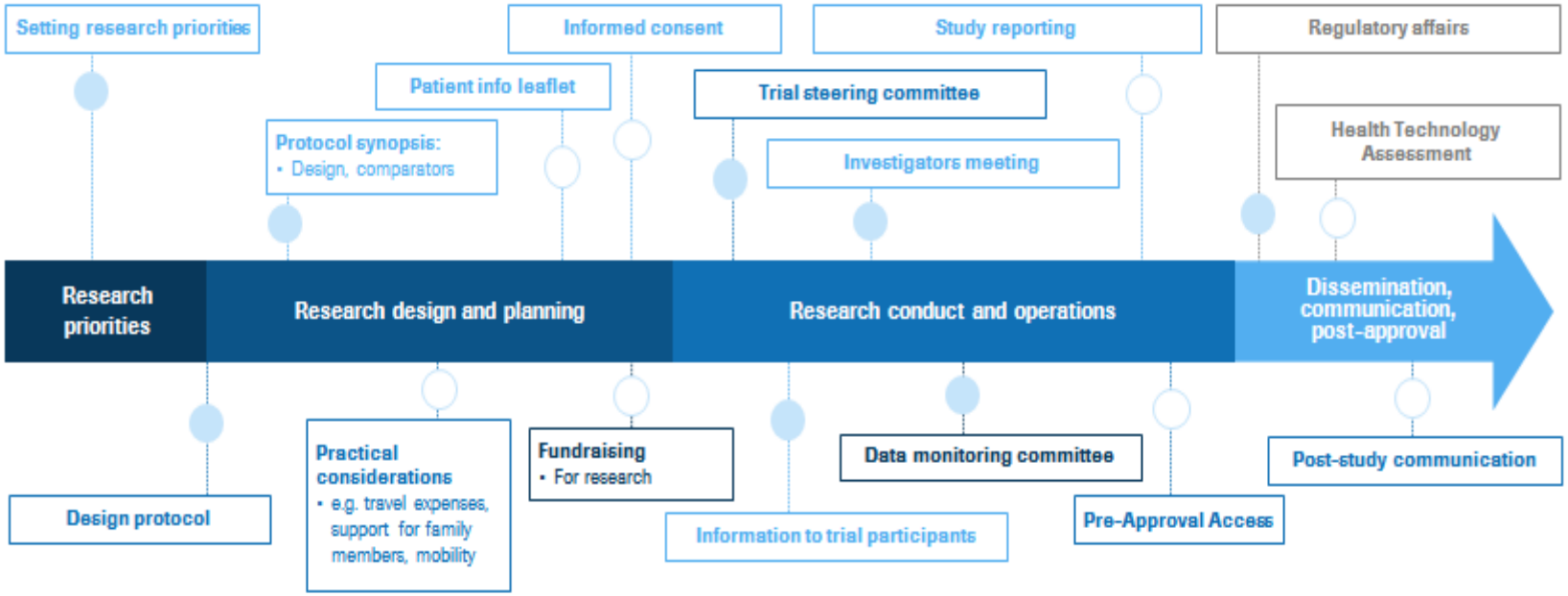
Were you short of breath?	1	2	3	4
Were you limited in doing your work or activities?	1	2	3	4

FDA 'clinical benefit': η επίπτωση της θεραπείας στο πως αισθάνεται, πως λειτουργεί και πως βιώνει ο ασθενής την πάθηση του

Συν-Διαμόρφωση του Κλινικού Προγράμματος και Συμμέτοχη σε όλο τον κύκλο ζωής του Φαρμάκου

Roche

Θεραπευτική Ανάγκη → Σχεδιασμό Μελέτης → Εκτέλεση Μελέτης → Έγκριση → HTA



Partnering with patients in our spinal muscular atrophy (SMA) clinical development program

Tim Seabrook[1], Kristina Geiblin[1], Ksenija Gorni[1], Thomas Wiese[1], Jeppe Buchbjerg[1], Anne Marquet[1], Christian Czech[1], Dominik Kraus[1], Dylan Trundell[2], Bertrand Verwee[1], Inge Schwersenz[3,4], Kacper Ruciński[3,5], Nicole Gusset[3,6], Serge Braun[7], John Baird[8], Karen Chen[9], Mary-Frances Harmon[8], Nikolai Naryshkin[8], Sergey Paushkin[8], Sangeeta Jethwa[1]

1. F. Hoffmann-La Roche Ltd., 2. Roche Products Ltd., 3. SMA Europe, 4. Deutsche Gesellschaft für Muskelkranke, 5. Fundacja SMA, 6. SMA Schweiz, 7. AFM Téléthon, 8. PTC Therapeutics Ltd., 9. SMA Foundation

Our SMA clinical development program consists of two investigational medicines given by mouth that are designed to work in different ways: oleso

Understanding the perspective of people with SMA* is important to us. We would like to thank everyone who provided input into our program.

Some examples are highlighted below.

Understanding what is important to people with SMA

Projects with input from people with SMA and caregivers

- Attending **our team meetings** to discuss unmet needs and challenges
- Creating an **SMA Independence Scale (SMAIS)** to capture **key impacts of Types 2 and 3 SMA** on independence and daily living
- Developing **conceptual models of disease.** These describe the experiences of living with SMA and may be used in future clinical trials
- Understanding what is meaningful for a new medicine helped us to prepare for **interactions with Health Authorities**
- Developing an **SMA App** to measure daily motor function, which may complement established assessments in clinical trials

*Use of the terms 'patients' and 'people with SMA' throughout this poster indicates all those affected by SMA, including patients, their families and caregivers.

Designing clinical trials

SUNFISH[1]
Children and young adults (2–25 years)
Type 2 or 3 SMA

FIREFISH[2]
Babies (1–7 months)
Type 1 SMA

JEWELFISH[2]
Children and adults (12–60 years)
Type 2 or 3 SMA

OLEOS[4]
Children and adults who previously participated in olesoxime[3] studies
Type 2 or 3 SMA

† RG7916 and olesoxime are investigational medicines.

Please refer to our poster 'Update on the Roche SMA clinical development program' for more information on these trials

All our clinical trials include feedback from people affected by SMA to select outcomes and minimize participation burden

- **Virtual advisory boards:** families of babies with Type 1 SMA reviewed the **FIREFISH** protocol
- **Expert patient review:** expert patients (people with SMA and experts in drug development) reviewed the **SUNFISH** protocol
- **Focus groups:** patient groups and people with non-ambulant Type 2 SMA advised us on the **OLEOS** study and the draft **Phase 3** study protocol for the investigational medicine olesoxime

Feedback has led to changes in our **study protocols and informed consent**

For more information on our SMA clinical development program, please visit **www.roche-sma-clinicaltrials.com**

References

1. https://clinicaltrials.gov/ct2/show/NCT02913482. Accessed April 2017; 2. https://clinicaltrials.gov/ct2/show/NCT02908685. Accessed April 2017; 3. https://clinicaltrials.gov/ct2/show/NCT03032172. Accessed April 2017; 4. https://clinicaltrials.gov/ct2/show/NCT02628847. Accessed April 2017.

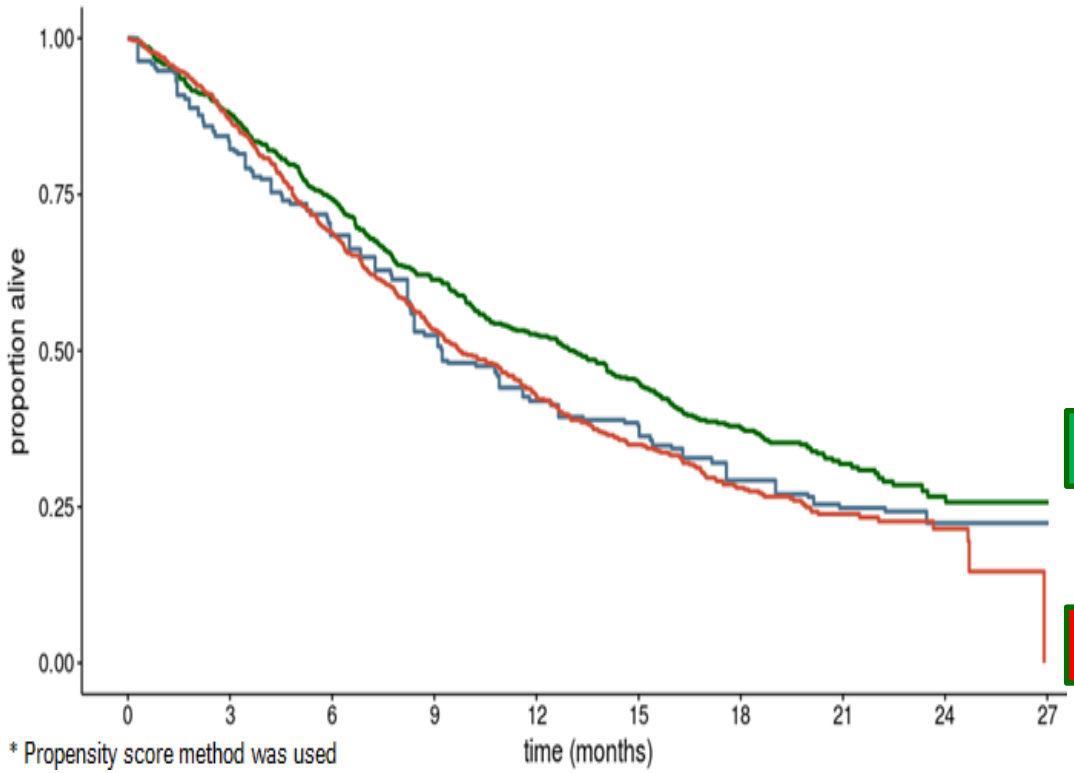
Acknowledgments

Thank you to everyone who has provided input into our SMA clinical development program. We would also like to thank CureSMA for helping to bring together all collaborators for the conceptual models project. Editorial support was provided by Sally Humphries of MedTech Media UK, funded by F. Hoffmann-La Roche Ltd. Genentech is a member of the Roche Group.

GPT/ESS2017/0078

- ***Develop the summary for a general public audience and do not assume any prior knowledge of the trial.***
- ***Develop the layout and content for each section in terms of style, language and literacy level to meet the needs of the general public.***

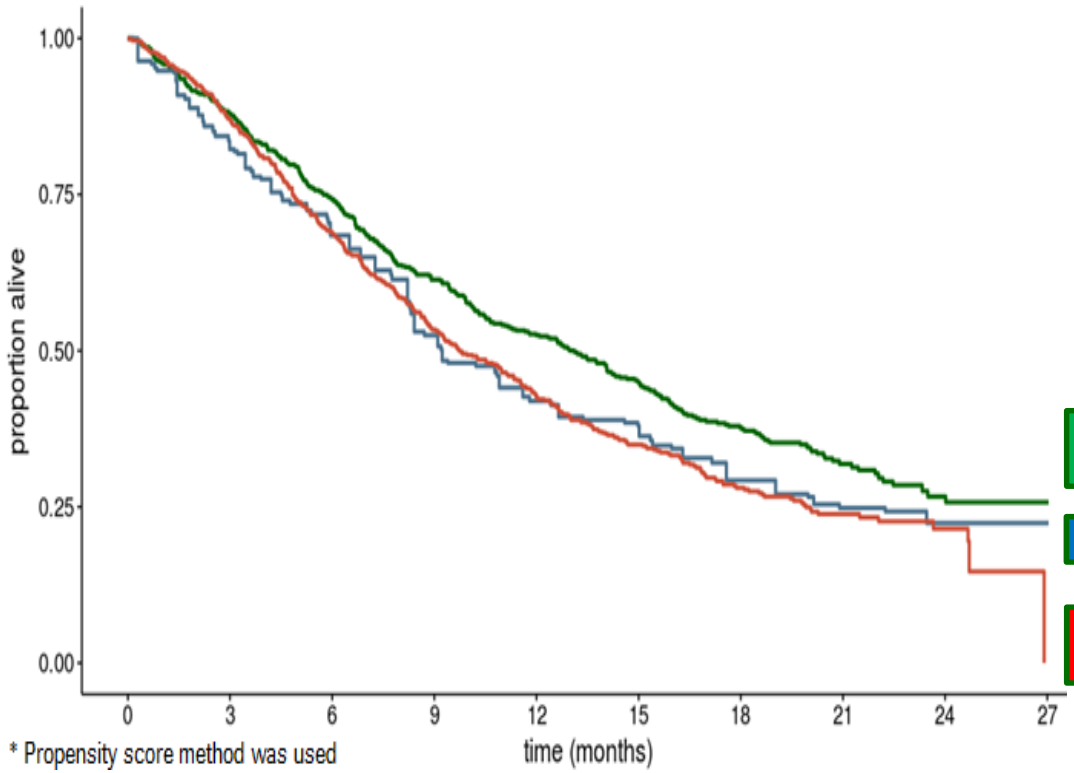
The Future of Research:



Νέο Φαρμακο-Εγκριτικη Μελετη

Παλιο Φαρμακο-Εγκριτικη Μελετη

The Future of Research: Virtual control arms



Νέο Φαρμακο-Εγκριτικη Μελετη

Νέο Φαρμακο από Registry

Παλιο Φαρμακο-Εγκριτικη Μελετη


INCLUSIVE RESEARCH
MISSION POSSIBLE

Το Μέλλον είναι στα «χέρια» μας!!!