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**“You told me to find an activity that I enjoy.
Challenging your authority is an
activity that I enjoy!”**







Specialist treatment centres



Specialist treatment centres
Research collaboration



Specialist treatment centres
Research collaboration
Access to drug funding



Specialist treatment centres
Research collaboration
Access to drug funding
Sharing knowledge and learning



Chordoma

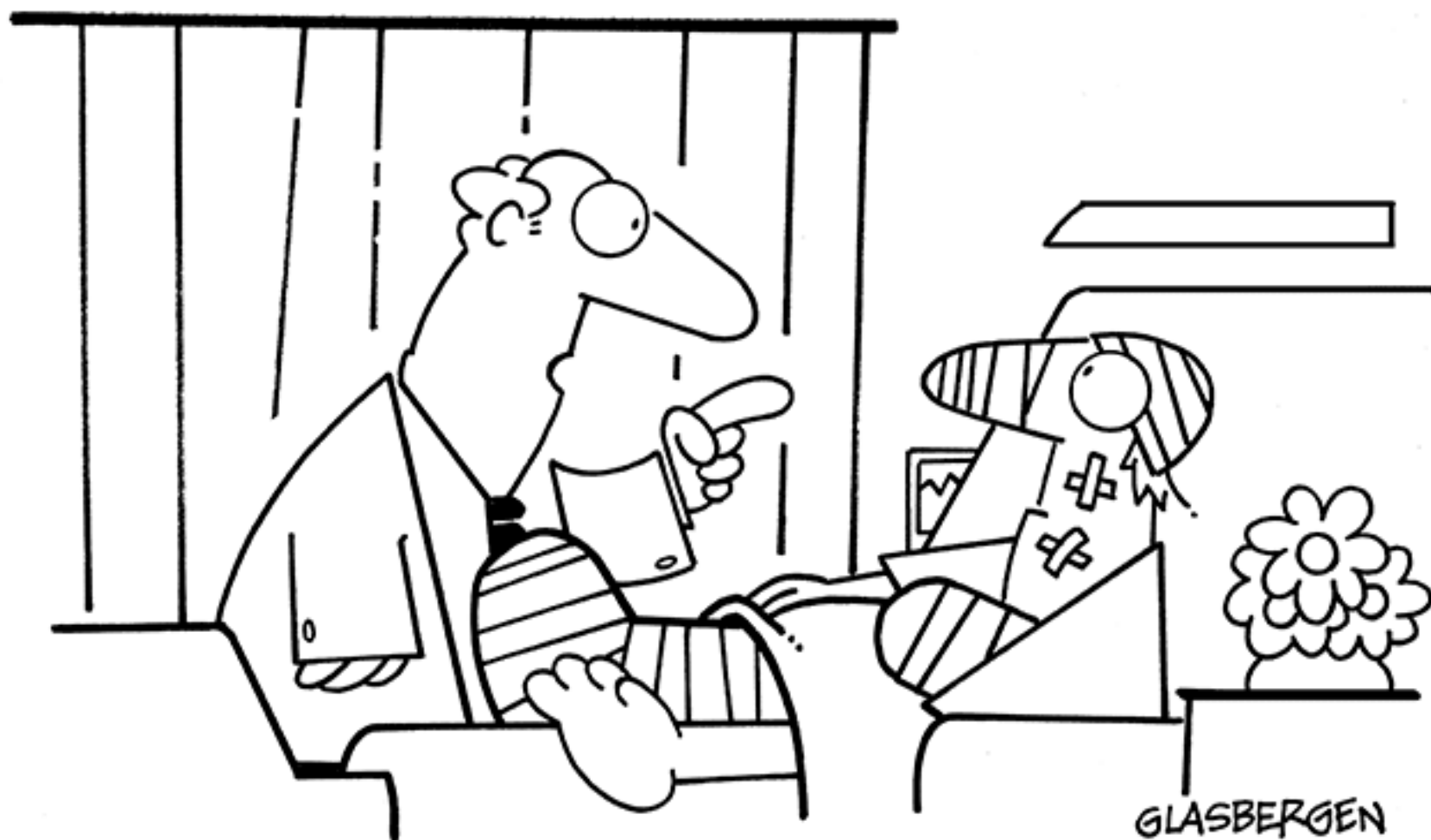
New Horizons

Wild-type GIST

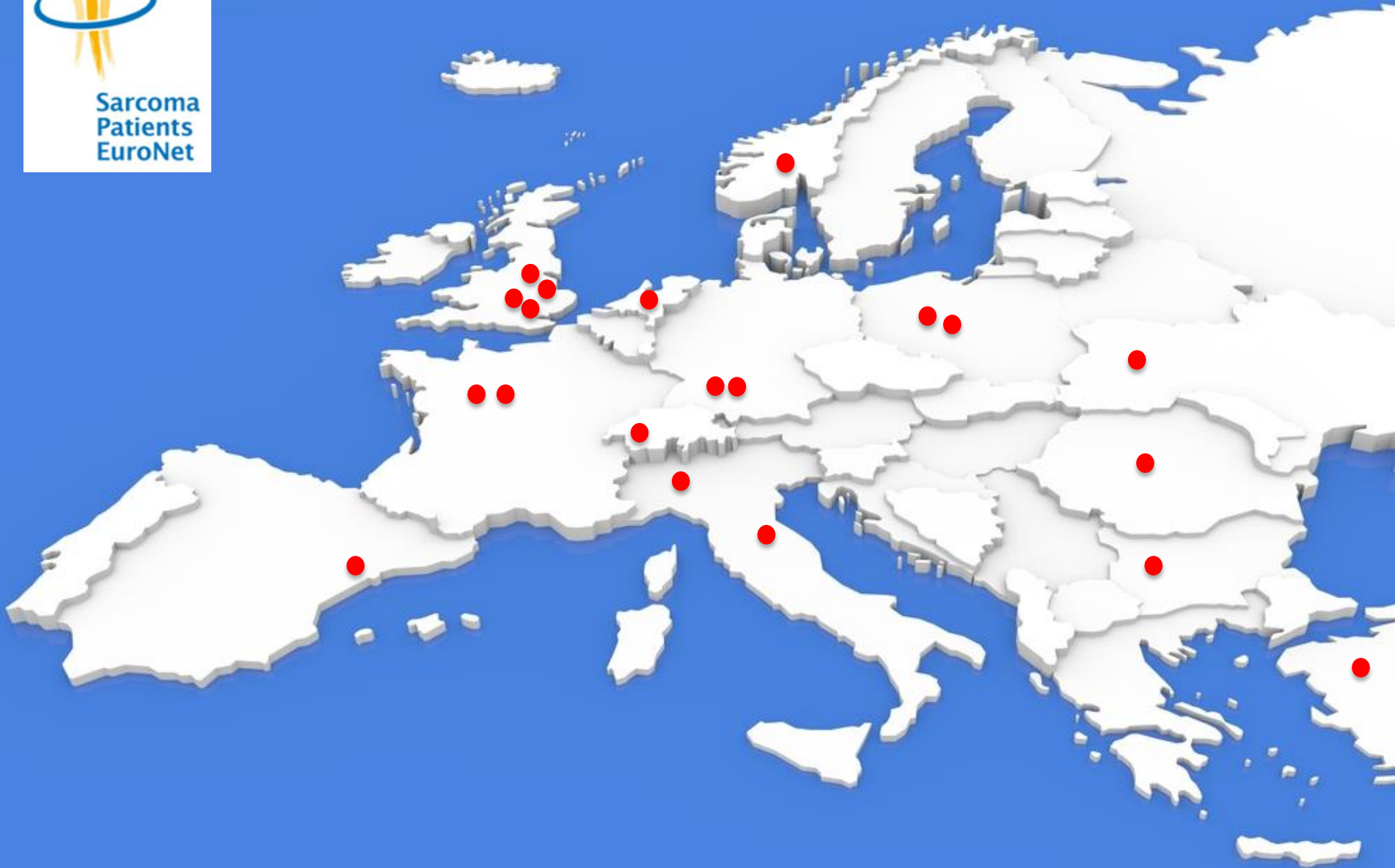
Desmoids



European and national regulators



**“Either lead, follow or get out of the way —
but never try to do all three at the same time!”**



The European Cancer Patient's Bill of Rights



Launch of **The European Cancer Patient's Bill of Rights** in Strasbourg on World Cancer Day, February 4th 2014.

The European Cancer Patient's Bill of Rights is the result of the **European Cancer Concord (ECC)**, an equal partnership between European cancer organizations and patients and their representatives who have come together to address the significant disparities that exist for European cancer patients. ECC is a patient-centered initiative, born out of the need to deliver an optimal standard of cancer care and research for Europe's citizens. Strengthening and upholding the rights of the individual cancer patient/cancer survivor are the guiding principles of this initiative.

ECC is dedicated to improving European cancer outcomes by seeking to enhance the patient's entire cancer journey, from prevention to early diagnosis, treatment, care, rehabilitation, patient survivorship and end of life care. It involves oncology and patient advocacy leaders from 17 European countries, who have come together in a unique partnership to address the inequalities in cancer care and research and to help develop effective solutions for European citizens and societies. To date, over 20 pan-European cancer Patient Advocacy Organizations (PAO), representing over 1,000 national organizations and many millions of cancer patients and survivors in Europe have become active partners.



EORTC

The future of cancer therapy





**“You have to learn about thousands of diseases, but
I only have to focus on fixing what’s wrong with ME!
Now which one of us do you think is the expert?”**

OBSERVATIONS

CLINICAL TRIALS

Subjects no more: what happens when trial participants realize they hold the power?

OPEN ACCESS

Patients will hold us all accountable in new and necessary ways

Paul Wicks vice president of innovation, Timothy Vaughan director of data science, James H cofounder and chairman

PatientsLikeMe, Cambridge, MA 02141, USA

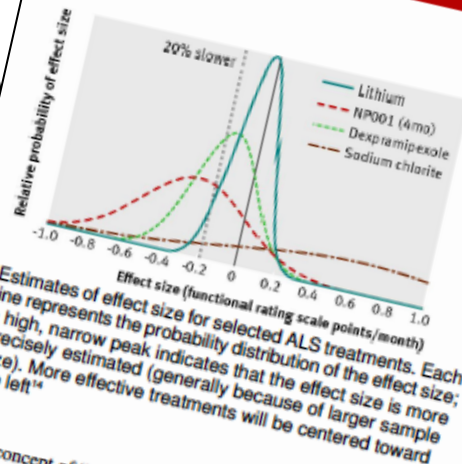
The social contract of the randomized controlled trial is imbalanced: patients adhere to arduous protocols, are randomized to placebo, and are blinded to their health status. Although most participants (>90%) would like a lay summary of results,¹ only a minority (<10%) receive one.² with the remainder left with the option of paying around \$30 (£18; €22) to read the results once the study is published in a peer reviewed journal.³ Such imbalances may have contributed to an emerging movement, enabled online by "patient powered research networks,"⁴ in which participants have begun systematically to unblind themselves, pool their data, parse literature, conduct statistical analyses, and post their findings online. In 2007, patients with amyotrophic lateral sclerosis (ALS) used Govele to translate an Italian conference abstract suggesting that a particular treatment might slow their illness.⁵ In a publication comparing 16 patients treated with dexampramipexole to 16 patients compared

randomized controlled trials subsequently reported findings.¹²

Shortly thereafter randomized controlled trials were under way: of NP001, manufacturer Biogen's dexampramipexole phase II trial) and Biogen's dexampramipexole. Participants in these trials shared data under protocols in which they were unaware of their ALSFRS-R score progress, seized on known side effects to attempt to unblind themselves to analyze the efficacy of both the NP001 group and 10% of US data online. A third experimenter read the patents on industrial cleaner sodium chlorate. Some patients who could industrial sodium chlorate although we had data

The concept of "scientific altruism" may be being trumped by "maximize your chance of survival." For better or worse, digital tools enable greater self knowledge and rapid dissemination. The consequence is that scientific design, informed consent, and ethical oversight can be short circuited by patient led clinical trial data (as AllTrials suggests), but even if they don't their data themselves, something that will only be enhanced by patient access to electronic medical records. Today members of PatientsLikeMe report tracking their outcomes in over 400 randomized trials. Patients increasingly realize that they are both statistically and literally the "power" in trials and we need to build systems that redress the imbalance. If we collectively do nothing, a phase III study might be rendered scientifically null by a critical mass of participants making intentional protocol violations on PatientsLikeMe, Facebook, or Twitter. This would be a tragic outcome. To prevent that, we propose forging a new social contract that maximizes both scientific discovery and patient autonomy, setting the stage for rigorous new methods.

reported outcome measures," which are now required by FDA.¹⁹ We believe that patients may surprise many of us with their ability to identify obstacles to trial enrolment, and the real world, not just in trials, and help us to understand the



Estimates of effect size for selected ALS treatments. Each line represents the probability distribution of the effect size; a high, narrow peak indicates that the effect size is more precisely estimated (generally because of larger sample size). More effective treatments will be centered toward the left.¹⁴

disposal patients will hold us all accountable necessary ways. Patients themselves have the groundwork; let's ask them to continue new systems together as equals.

Competing interests: We have read and understood declaration of interests and declare PW, TV, and JH PatientsLikeMe and own stock or stock options in the PatientsLikeMe research and development team has support from pharmaceutical companies and private fund. Provenance and peer review: Not commissioned; not reviewed.

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**"My cholesterol pills are easier to swallow
when I sauté them in butter."**

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