Roger Wilson

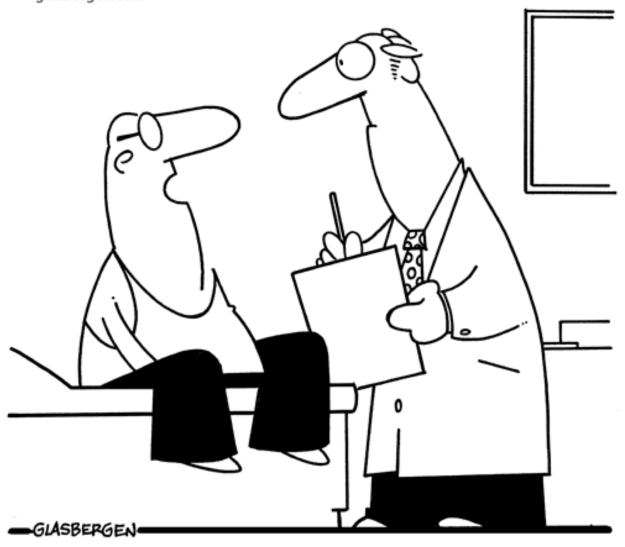
Hon President Sarcoma UK President Sarcoma Patients Euronet

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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

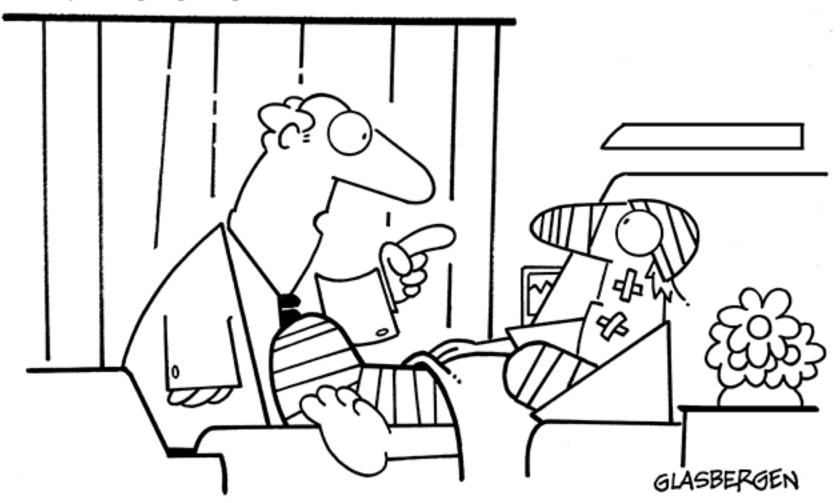
New Horizons

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 Right

Strasbourg on World Cancer Day, February 4th 2014. European Cancer Concord (ECC), an equal partnership between European to address the significant and patients and their representatives who have come together to address the significant and patients and their representatives. The European Cancer Patient's Bill of Rights is the result European Cancer Concord (ECC), an equal partnership between European 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 out or the fleed to deliver an optimal standard or cancer care and research for Europe's citizens. Strengthening and upholding the rights of the individual cancer patient/cancer

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





"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?"



Page 1 of 2

BMJ 2014;348:g368 doi: 10.1136/bml-g368 (Published 28 January 2014)

OBSERVATIONS

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

tit

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 nnounneer, panents annere to autrous protectis, 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 or results, only a minimity (<10%) receive one, with the region 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, of in which participants have begun systematically to networks, in which participants have begun systematically it unblind themselves, pool their data, parse literature, conduct

statistical analyses, and post their findings online. In 2007, patients with amyotrophic lateral sclerosis (ALS) used Goorle to translate an Italian conference abstract suggesting an amount conserved available augicus augentation of ALS" 16 patients treated

randomized controlled trials subsequently rep

Shortly thereafter randomized controlled to findings.12 were under way: of NP001, manufactury phase II trial) and Biogen's dexpramip Participants in these trials shared data under protocols in which they were unaware of their ALSFRS-R score progress, seized on known side es an attempt to unblind themselves. to analyze the efficacy of both 9 NP001 group and 10% of US 9 data online. A third experime patients read the patents on industrial cleaner sodium o Some patients who could industrial sodium chlor whough we had data

20% slower ! - Lithium --- NP001 (4mo) ---- Dexpramipexole --- Sadium chlarite

BMJ 2014;348:g368 doi: 10.1136/bmj.g368 (Published 28 January 2014)

-0.6 -0.4 -0.2 0 Estimates of effect size for selected ALS treatments. Each 0.2 0.4 0.6 0.8 1.0 Estimates or 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 a riigit, flarrow peak irioicales that the effect size is more precisely estimated (generally because of larger sample precisely estimated (generally because of larger sample stands of the sample of the sample of the sample stands of the sample stands of the sample sa

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 disobedience." Some drug companies will choose to share their clinical trial data (as AllTrials suggests), but even if they don't the data can become available if participants choose to share their data themselves, something that will only be enhanced by patient access to electronic medical records, Today members patient access to encuronic metural records, recay memoris of Patients LikeMe 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 sciencis discovery and patient autonomy, setting the trials with more engaged particip rigorous new method

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

Competing interests: We have read and understoo declaration of interests and declare PW, TV, and Ji-PatientsLikeMe and own stock or stock options in th PatientsLikeMe research and development team has i support from pharmaceutical companies and private for Provenance and peer review: Not commissioned; not e

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progression using Google spreausiness functional rating scale (ALSFRS-R). A patient in biassis patients reported ona caregiver in the United States initiated this patient led study, raising the question of where ethical oversight lay. Our patient network, PatientsLikeMe, already allowed entry of

to draw a relia ingesting off expected (fi effect,16 an When AL dangers

access to patients for concept elicitation reported outcome measures, " which are now required by the FDA. 19 We believe that patients may surprise many of us with their ability to identify obstacles to trial enrolment the outcomes they truly value, and balance the real world, not inet in trial

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

Roger Wilson

Hon President Sarcoma UK President Sarcoma Patients Euronet

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