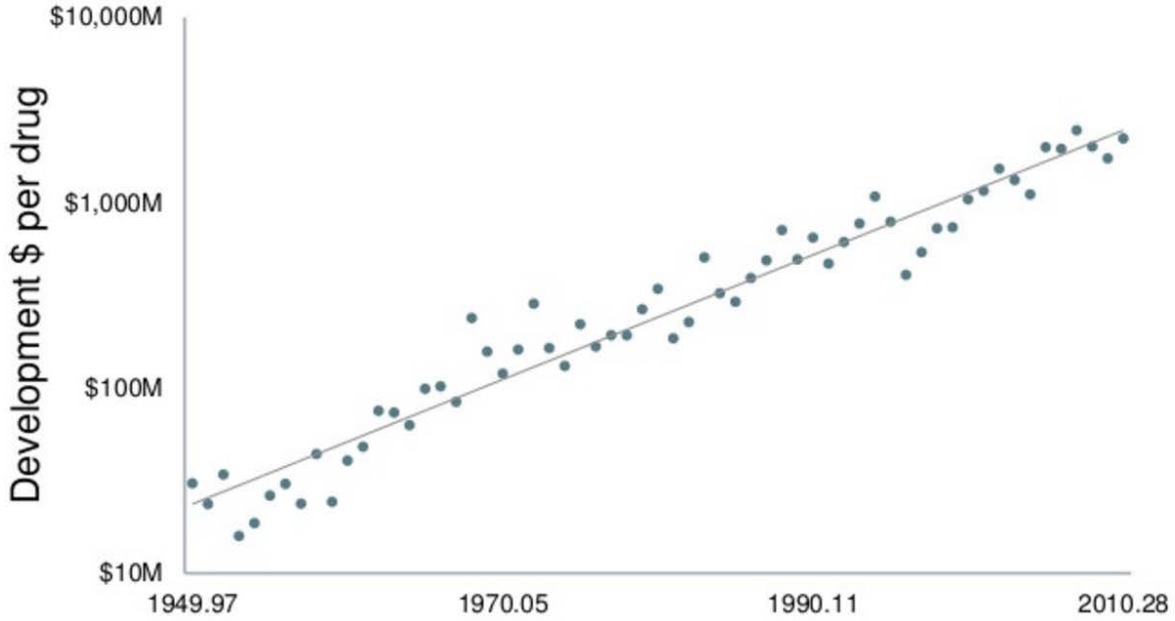
OSLO CANCER CLUSTER

FROM CANCER RESEARCH TO CURE

Dedicated to accelerating the development of new cancer treatment

BLIR STADIG DYRERE Å FINNE NYE MEDISINER

Eroom's law: $/drug exponentially increasing



Source: Nature Biotechnology

ANDREESSEN HOROWITZ

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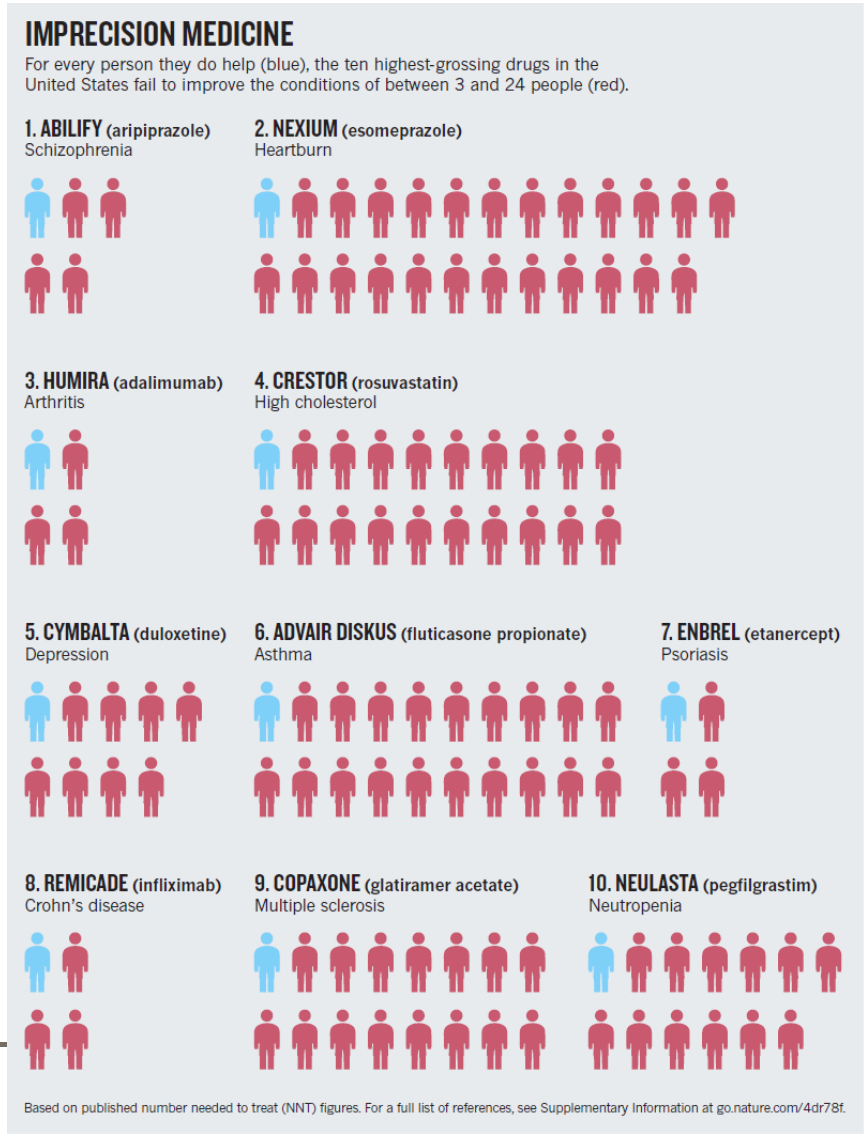

nature
International weekly journal of science

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TILGJENGELIGE HELSEDATA ENDRER MÅTEN VI BRUKER MEDISINER

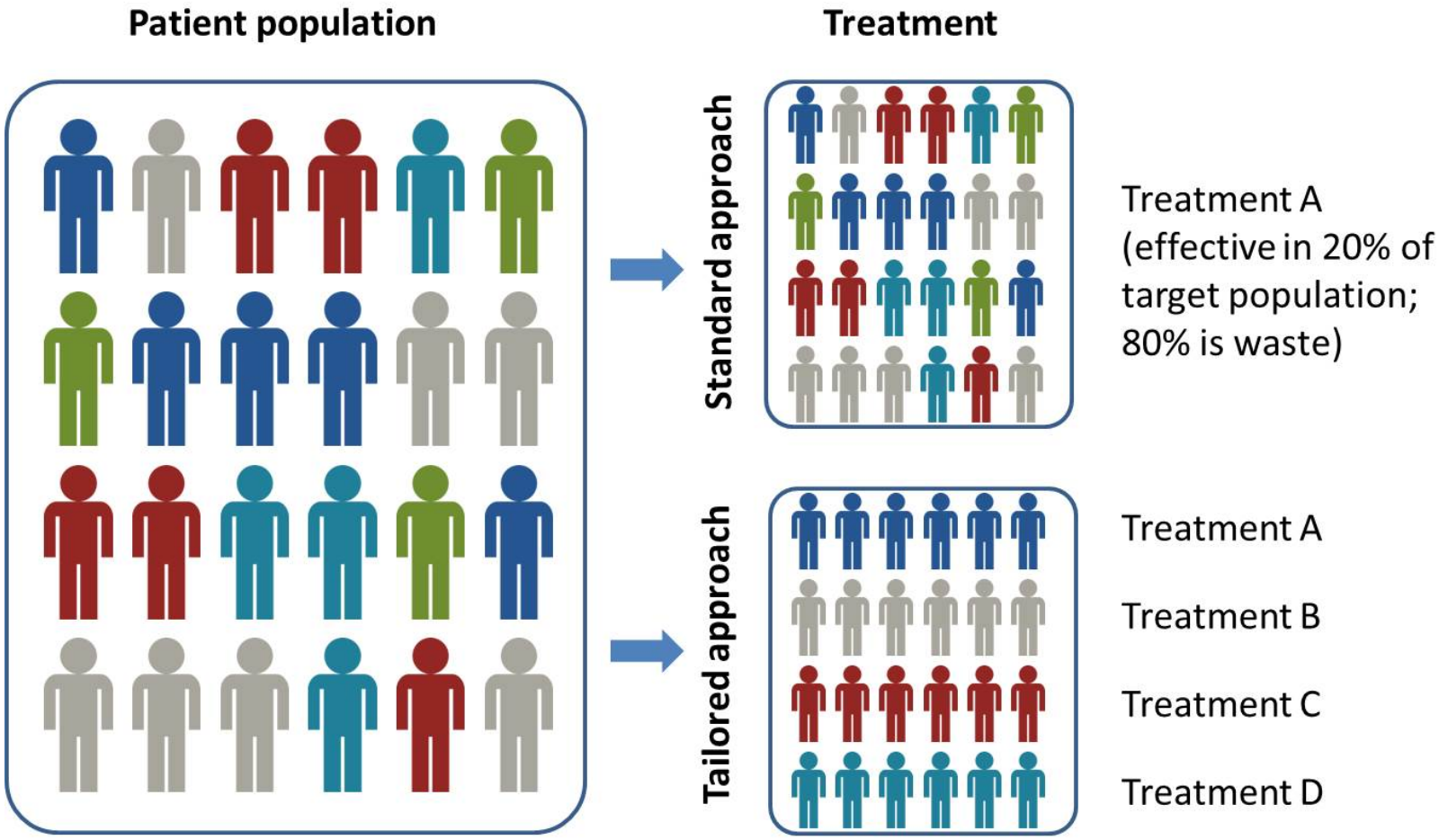
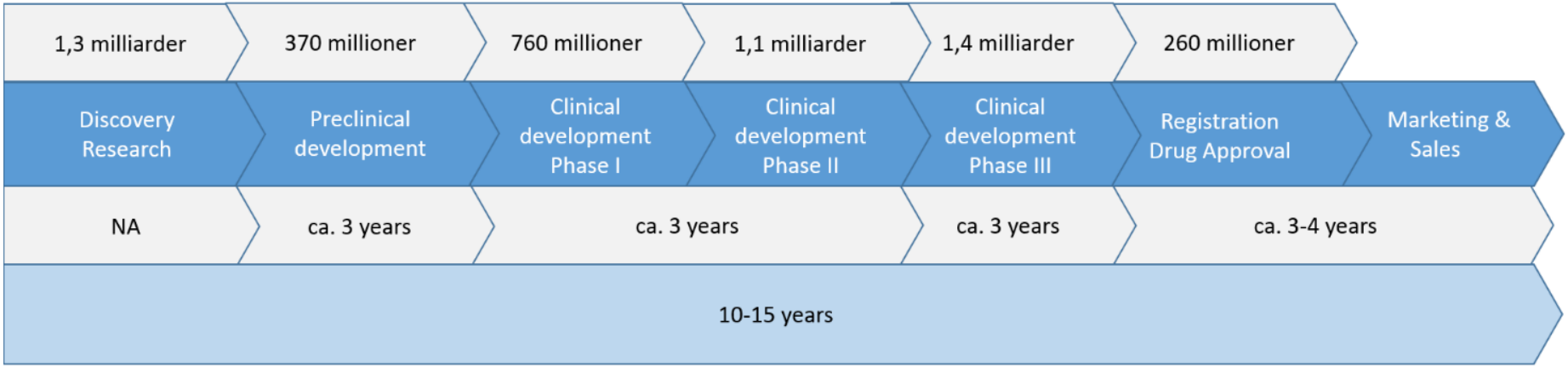


ILLUSTRATION BY GREG CLARKE

Time for one-person trials

Precision medicine requires a different type of clinical trial that focuses on individual, not average, responses to therapy, says **Nicholas J. Schork**.

SYSTEM FOR GODKJENNING
LAGET FOR STORE PASIENTGRUPPER



- **Phase I:** evaluate drug safety and tolerability; how the body absorbs and separates the drug, whether it is toxic, and whether the drug has an effect/side effects. Tested in app. 20-150 humans.

- **Phase II:** looks at the medical effects of the drug in patients. Determine when, how, and in what quantities (doses) the drug should be given, and document the most common side effects. Tested in app. 100-200 patients.

- **Phase III:** therapeutic confirmatory phase, seeks to confirm that the drug is safe and effective by the disease and patient group, often compared with the current standard treatment. Tested approximately 100-5000 patients.

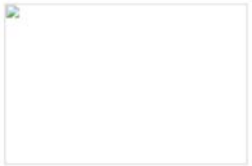
LEGEMIDLER I BRUK FØR FASE 3; EKSEMPLER, NÅ TEKNOLOGISK MULIG

MAY 4, 2012 @ 10:25 AM 9,139 VIEWS

Should the FDA Approve More Drugs after Phase II
Matthew Herper

Avik Roy,
Political commentary from Forbes' Opinion Editor FULL BIO
Opinions expressed by Forbes Contributors are their own.

Last Friday, *Forbes* health care editor Matt Herper and I sat down to talk about my proposal, which I detailed in a paper for the Manhattan Institute, to encourage the FDA to approve more drugs after mid-stage phase II testing, using a process called "conditional approval." (You can read my proposal, in three parts, here.) Matt put forth some very perceptive critiques of the idea, which I respond to in today's dispatch.



US Secretary of Health and Human Services Kathleen Sebelius (R) speaks alongside Food and Drug Administration (FDA) Commissioner Margaret Hamburg during the Daily Press Briefing in the Brady Briefing Room of the Wh House in Washington, DC, June 21, 2011. (Image credit: AFP/Getty Images via @daylife)

As a refresher, my proposal builds on an existing FDA procedure called *accelerated approval* in which the FDA approves drugs that show great promise in phase II, with the caveat that the drug sponsor must still perform confirmatory phase III studies. If the phase III studies ultimately show that the drug doesn't work as advertised, or has previously unknown safety issues, the FDA can revoke its approval. This is exactly what happened when the FDA revoked the approval of Avastin in breast cancer, after phase III tests did not reproduce the early signal of benefit that the drug had shown in phase II studies.

The Oncologist

Editorial

Approval After Phase I: Ceritinib Runs the Three-Minute Mile

BRUCE A. CHABNER
Massachusetts General Hospital Cancer Center, Boston, Massachusetts, USA
Disclosures of potential conflicts of interest may be found at the end of this article.

Bruce A. Chabner

Thirteen years ago, I wrote an editorial applauding discovery, development, and marketing approval of imatinib for chronic myelogenous leukemia (CML), a signal event in the history of targeted therapy, and the oncology equivalent of the four-minute mile [1]. It took 3 years from the start of trials and required the confirmatory evidence of two phase II studies to receive the U.S. Food and Drug Administration's (FDA) stamp of conditional approval. A decade later, these pages called attention to the rapid 3-year development of crizotinib for ALK-translocated non-small cell lung cancer (NSCLC), again based explained by its greater potency and its particular ability to inhibit ALK with gatekeeper mutations that confer resistance to crizotinib. In this trial, mechanisms of resistance were characterized in a subset of 19 crizotinib-resistant tumors prior to ceritinib treatment, and responses to the new drug were observed in settings where gatekeeper mutations were present, where ALK was amplified, or where no obvious mechanism was identified. While activation of alternative pathways is suspected of contributing to resistance, particularly when tumors fail to show amplification or mutation

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BY TIMOTHY GOWER //
ART BY CHRIS GASH

PUBLISHED ON SEPTEMBER 28, 2017

POLICY

Phasing Out Phase 3

What if drugs were released to the public earlier, then graded on their performance in the real world?

The cry for *more drugs, more quickly* comes from both sides of the political aisle. It sped the bipartisan passage of the 21st Century Cures Act in 2016—sweeping legislation that, among other things, aimed to streamline drug and device approval. And in his first address to Congress, President Donald Trump spoke of the "slow and burdensome" approval process of the Food and Drug Administration and recommended "we slash the restraints."

How that might be done, and whether it should be done, is a matter of much debate. One of Trump's early candidates for commissioner of the FDA was Jim O'Neill, a former official at the U.S. Department of Health & Human Services, who called for releasing drugs to the public after they satisfied only basic safety requirements. Rather than miring new drugs in expensive clinical trials, he said, which can last years and cost millions of dollars, "let's prove efficacy after they've been legalized."

That idea is controversial, but not without notable adherents. Most drugs currently go through a three-phase approval process, with phase 1 focusing primarily on safety using a small number of people. Phase 2 tests safety and effectiveness, generally through trials in which some patients get the drug and others receive the current treatment or a placebo. Phase 3 also uses control groups and measures how well the drug works in larger populations. But as long as a drug appears safe in a phase 2 study, the argument goes, why not release it then? Instead of going through phase 3, drug companies can monitor patient data—real-world evidence, or RWE—to see if the drugs work.

The NEWDIGS (for NEW Drug Development ParadIGmS) "think and do" tank at the MIT Center for Biomedical Innovation has been putting meat on the bones of this idea. "We think we can do better

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I NORGE ER IKKE ALT PERFEKT…

ONSDAG 22. NOVEMBER 2017

Tidsskriftet
DEN NORSKE LEGEFORENING

Norske helsedata – en utilgjengelig skatt

LEDER | ALLMENNMEDISIN

Knut Erik Emberland, Guri Rørtveit *Om forfatterne*

Publisert: 10. oktober 2016

No. 18, 11. oktober 2016
Tidsskr Nor Legeforen 2016
136:1506
DOI: 10.4045/tidsskr.16.0613

Forskerens vei til helseregistrene er kronglete, tidkrevende og kostbar. Dette gir unødvendig risiko for befolkningens helse.

Norske helsedata omtales gjerne som en gullgruve for forskere. Norge har i dag 16 sentrale helseregistre. I tillegg regnes opplysninger fra de befolkningsbaserte helseundersøkelsene, biobanker og kvalitetsregistre som helsedata. Sammen med våre unike personnumre gir disse datakildene muligheter til å avklare medisinske spørsmål på en måte som er mulig i få andre land. Norske forskere er gode til å belyse problemstillinger med slike data, med kunnskap formidlet gjennom artikler i verdens ledende tidsskrifter som resultat (1 – 3).

«Helsedata som nasjonalt fortrinn» er et av HelseOmsorg21-strategiens ti satsingsområder (4). I samme strategi er «Lettere tilgang til og økt utnyttelse av helsedata» én av fem hovedprioriteringer. Det er betimelig, for veien til helsedata er

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… MEN VI KAN TA LEDELSEN OM VI SATSER

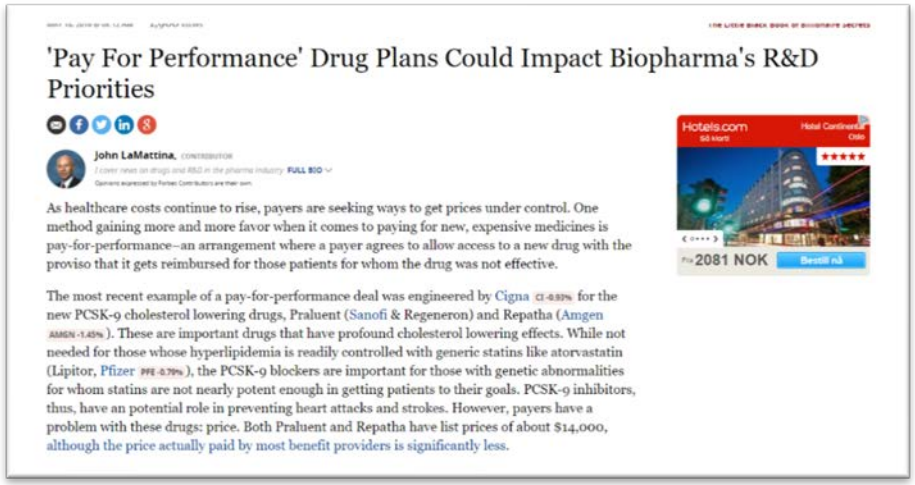
- Et helsesystem og individuelle personnummer
- Lite mobilitet i befolkningen
- Nasjonale kliniske biobanker
  - E.g. National Cancer Genomics Consortium
- Nasjonale kvalitetsregistere
  - E.g. Kreftregister over 60 år

→ Norge posisjonert for å bli et globalt senter for utvikling og testing nye medisiner

RAPPORT ANBEFALER BRUK AV OFFENTLIGE HELSEDATA TIL DOKUMENTASJON

- Opprette en instans, tilgangsforvalter, som er ansvarlig for tilgjengeliggjøring og markedsføring av helsedata,
- Realisere helseanalyseplattformen rask – denne vil gi tilgang og samtidig sikre personvernet
- Avvikle dagens krav til forhåndsgodkjenning som i enkelte tilfeller forsinker prosjekter med flere år
- Gi muligheten å bruke helsedata som dokumentasjonsgrunnlag for raskere og bedre godkjenning av legemidler.

NORGE PILOTLAND FOR REGISTERBASERTE FASE 3?



→ billigere, raskere og mer presise legemidler

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UCSF

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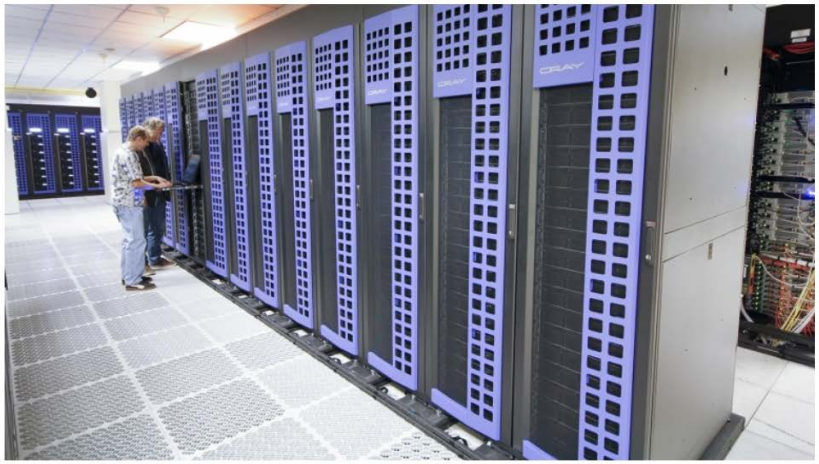
Public-Private Consortium Aims to Cut Preclinical Cancer Drug Discovery from Six Years to Just One

Lawrence Livermore National Laboratory, Frederick National Laboratory for Cancer Research, GSK, and UCSF Partner on Effort

By Laura Kurtzman on October 27, 2017



A supercomputer in the Lawrence Livermore National Laboratory, which will be used in a new public-private project to speed discovery of new drug therapies. *Photo by Lawrence Livermore National Laboratory*

Scientists from two U.S. national laboratories, industry, and academia on Oct. 27 launched an unprecedented effort to transform the way cancer drugs are discovered by creating an open and sharable platform that integrates high-performance computing, shared biological data from public and

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