Second Annual Medical Research Summit

The Context of Clinical Research: Challenges and Opportunities

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"I have always known, that
At last, I would take this road,
But yesterday, I did not know,
That it would be today."

- Narihira, 1000 years ago

• Human beings have long hoped and believed that at some point we would unlock the mysteries of how life works; we are almost overwhelmed to be living in the time when we are beginning to do just that.

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• In this era, many scientific discoveries and highly innovative life-saving and enhancing therapeutics are developed through biomedical research involving human volunteers.

• Over the past two years, *Newsweek* magazine coverage has illustrated that medicine is changing because of the Human Genome Project and other scientific and technological advances at academic medical centers, biomedical companies and research organizations in the U.S. and abroad.

The Next New Thing

A revolution in genetic research is targeting treatments to patients' unique characteristics. It can mean the difference between life and death. By Sharon Begley

Made-to-Order Medicine

JILL WAS ONLY 2 WHEN the diagnosis came: acute lymphoblastic leukemia (ALL). This rare childhood cancer, the doctors assured her parents, is highly curable with a cocktail of four chemotherapy drugs. But from the very beginning the chemo made Jill acutely ill: her white-cell, red-cell and platelet counts plummeted, and even with biweekly transfusions "her counts kept going lower and lower," says Dr. Mary Relling of St. Jude Children's Research Hospital in Memphis, where Jill was treated. Doctors didn't know whether the leukemia was knocking out her blood production—or whether the chemo itself was. But they had a way to find out. Researchers at St. Jude and at the Mayo Clinic in Rochester, Minn., had recently discovered that patients with a single mistake in a gene called TPMT fail to produce the enzyme that metabolizes the chemo drug, 6-mercaptopurine. As a result, the drug builds up in the body to toxic levels. Jill belonged to the 0.3 percent of the population—one person in 300—that carries two copies of the misspelled TPMT

SPECIAL TREATMENT: Herceptin, a drug developed by Dr. Dennis Slamon, targets a receptor found in only 30 percent of breast cancers

New technology will help you monitor your health, treat your phobias, screen your blood, check your moles and scope out your system. A sampler:

Your Next...

Smart Shirt

THE NAUTICAL LOOK IS hot, but next season's Navy-inspired shirts may be more high tech than high style. Georgia Tech engineers, sponsored by the Navy, have developed a Smart Shirt that can monitor your vital signs and beam urgent messages to your doctor. In combat, the shirt could be a lifesaver: it can detect bullet wounds with optical sensors and radio back to HQ for help. Within the next five years, doctors may use it to keep an eye on post-op patients, athletes and astronauts. And for new parents worried about SIDS? Tiny wired pajamas.

Bandage

ATTENTION, MOMS AND dads: when Junior comes home with skinned knees, a smart bandage may keep him out of more serious trouble. Sensors in a bandage developed by the University of Rochester can screen for bacteria or viruses in a wound, signaling the need for antibiotics. Pacific Northwest National Laboratory is working on its own smart bandage, which would help cure chronic wounds by ridding the sores of an enzyme that breaks down proteins and hampers healing. Still another bandage from the University of Virginia sucks oxygen-rich blood to damaged tissue to speed up recovery.

Wireless Endoscope

DOCTORS PERFORM ABOUT 2 MILLION ENDOSCOPIES a year on the small intestine, searching for cancers and other ailments by inserting fiber-optic tubes up the rectum or down the throat. This can be painful, and some areas remain inaccessible. But if the FDA approves Given Imaging Ltd.'s "wireless video pill," some patients may be cruising around this fall while the M2A passes painlessly through the system. For 24 hours it snaps two pictures a second, beaming images to a small recorder worn at the waist; the data are downloaded to hospital computers for analysis. The capsules won't banish the endoscope, which also can perform biopsies and treat disorders. "But ... it's going to be a leap forward," says Dr. David Lieberman of the American Society for Gastrointestinal Endoscopy. The capsules are disposable, of course.

Blood Screening

SCIENTISTS CALL IT TNV—THE NEXT virus. It's the one that could be percolating in blood banks this moment. But Cerus Corp. may have created the first defense shield for the world's blood supply. Its Helinx compounds bind with DNA and RNA, inactivating all viruses and bacteria, including HIV and hepatitis. (Healthy blood components are unaffected.) The filter is not a cure for people already infected with HIV—the virus replicates too quickly and lodges itself in bodily tissues—but it makes transfusions safer and could increase blood supplies once banks trust the technology. Cerus hopes to gain FDA approval late next year.

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• As with every scientific and technological breakthrough, it is not just the discovery itself that is important, but how we use it.

• The stakes are even higher now, as unlocking the genetic codes for human beings and other species empowers us to rethink who we are and to change both ourselves and our environment.

• Given the gift of this great opportunity to alleviate suffering and improve the human condition, we must rise to the challenge of conducting our research—and especially our clinical research involving human participants—in accordance with the highest ethical and scientific principles.

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- We must embrace and adhere rigorously to best practices performance standards in medical research. This is essential to ensure scientific outcomes that can be translated into better medicine. It also is essential to maintain and enhance public trust, the backbone of continued progress.

- This session will explore why quality clinical research and development in academic medical centers is and must be regarded as a business-value proposition. Academic leaders, public and private research investors, research outcomes users (e.g., Big Pharma), regulators and the general public no longer will accept anything less.

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Reviewing the Basics: It's a New Era in Medical Research

- Expansion in our scientific knowledge base, with associated progress in biomedical and life sciences research and applications.
- Greater availability of intellectual and financial capital devoted to biomedical and life sciences research and development.
- Intellectual property has become a primary means through which research developments are recognized as advances, used in further research and translated into downstream applications.
- Highly publicized research compliance failures at leading academic medical centers have threatened public trust in and support for clinical research.

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Reviewing the Basics: It's a New Era in Clinical Research (Continued)

- Federal officials have stepped up oversight and enforcement, and are endorsing a new research paradigm that emphasizes risk management and voluntary accreditation benchmarked to stricter standards.
- Increased exposure to civil and class-action litigation stemming from regulatory compliance failures during both R&D and post-approval marketing.
- Need for an integrated systems approach to measuring, monitoring, improving and reporting compliance to public and private research investors, research outcomes users, regulators and other stakeholders.

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Keys to Research Success in this New Environment:

- Funding
- Publications
- Patents

Levers:

- Fear of Enforcement and its Consequences.
- Potential Organization and Intellectual Property Value Enhancements.

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A New Era Requires a New Way of Thinking About Research Compliance:

- Current State: Primary goal of compliance is avoidance of enforcement by managing and minimizing compliance compromises and failures;
- Better State: Primary goal of compliance becomes organization enhancement through compliance linked to best practices performance standards, with enforcement avoidance as an integral result.
- THE BOTTOM LINE: Research projects and outcomes must be regarded as "investment grade," worthy of funding, patents and publications. The market will demand documented excellence across the board. "Getting by" no longer makes the grade, whether in the private sector or at academic medical centers.

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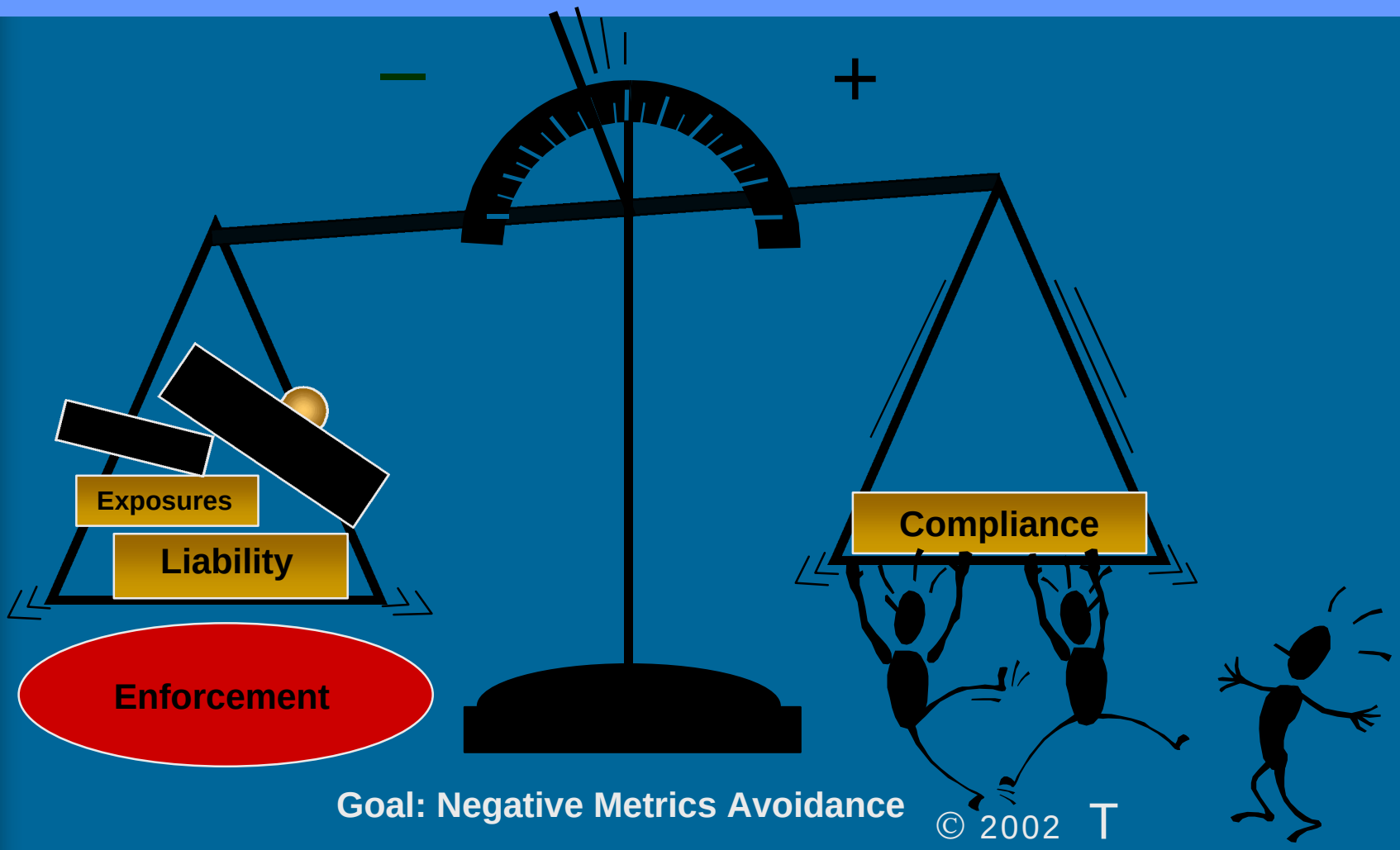
Current State



Goal: Negative Metrics Avoidance

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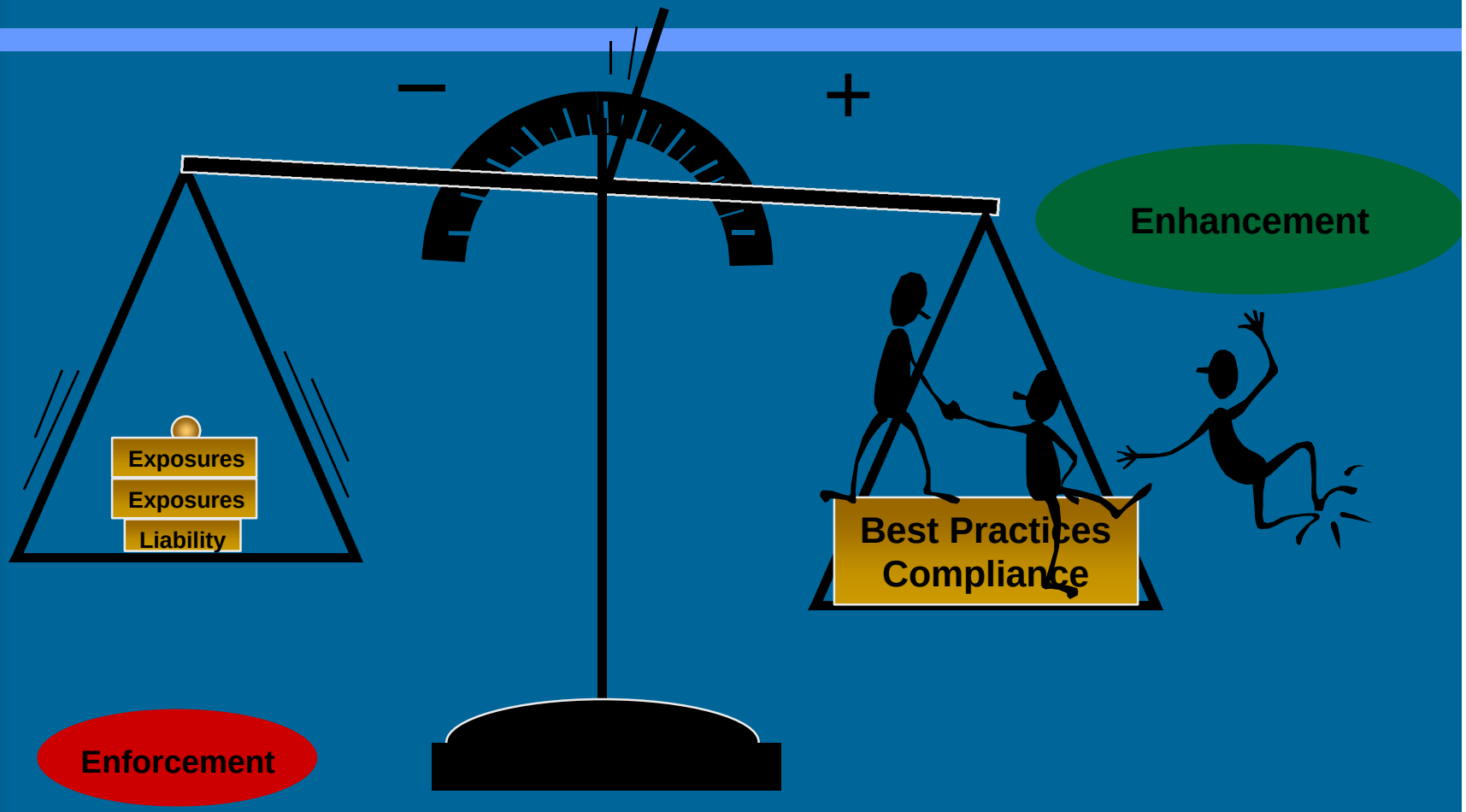
Better State



Goal: Positive Metrics of Better Regulatory/IP Compliance and Higher IP Portfolio Value, Security and Transferability

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Benefits and Payoffs:

- More prestigious, attractive and secure research environments for public and private research sponsors.
- Improved organization performance.
- Ability to document and demonstrate research and development compliance to government grant-making and regulatory agencies, research outcomes users, the investment community and the general public.
- Proactive self-protection against the expansion of anti-fraud and abuse regulatory initiatives into biomedical and life sciences research and development.

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Benefits and Payoffs (Continued):

- Assurance that scientific insights and discoveries of enormous therapeutic potential and market value are identified rather than missed or lost during the research process.

AND…

- Ability to identify, protect, better value and leverage quality academic research in leading intellectual and commercial capital marketplaces.