Remarks by the Acting Commissioner of Food and Drugs

NORMAN SHARPLESS, MD

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Thank you Jennifer [Bragg] for that introduction. And thank you also Amy [Comstock Rick] for all the work you do running this organization. I'm delighted to be with all of you today.

FDLI is an important organization that has long served as a key partner and advocate for FDA's work.

I use the word "advocate" here not because I'm standing in a room with so many lawyers, or because you always agree with everything we do at FDA. (In fact, I doubt very much whether we could ever get a group of lawyers to agree on everything on just about any topic . . . .)

No, I use the word advocate to describe your relationship with FDA and because of your keen understanding of the FDA's mission.

Your expertise and engagement on the broad range of issues we address reinforces and strengthens our efforts to use science in the development of law and policies that make a meaningful difference to the health and safety of the American public.

I will confess to a small amount of trepidation—as a doctor and cancer researcher—about talking to a group of so many lawyers. I speak doctor; I don't really speak lawyer. Despite differences in language, however, I think the medical and legal professions share many important qualities. The relationship of a doctor and patient is like that of a lawyer and client. In both cases, the relationship is one of service.

At the FDA, that patient or client, if you will, is the public health of Americans.

Perhaps I won't be able to fully bridge the doctor-lawyer divide for this audience, but in that case I also came prepared. Later today, after lunch, you'll be hearing from FDA's top lawyer, Stacy Amin, and I'm sure she'll be able to advance some of the same points I'm making in appropriate legalese—with requisite *ipso factos* and *nolo contenderes* as needed.

FDLI AND THE FDA/MY BACKGROUND

We at the FDA take seriously the opportunity to be a part of this annual conference.

The Commissioner's annual remarks to FDLI have particular significance, offering an opportunity to reflect on the Agency's accomplishments over the past year, and to chart our path moving forward.

The timing this year for me couldn't be better. I've been on the job now closing in on one month, and I'm glad to have the opportunity to introduce myself.

Let me say out the outset that I don't anticipate extraordinary departures from the FDA's recent priorities and course of action.

Given the breadth of the FDA portfolio, taking on the role of Acting Commissioner is daunting. But I feel up to the task because of the great and stable leadership at the FDA who provide advice, and because of my relevant background.

As you may know, I've been the director of the National Cancer Institute since 2017. Before that, I was a cancer researcher and cancer doctor, treating patients with hematologic malignancies, for nearly twenty years in academia.

During that time, I ran a successful NIH-funded lab studying the molecular mechanisms of cancer and aging, and I also was director of a large Comprehensive Cancer Center at the U of NC.

I've been involved in helping patients through the clinical development of novel diagnostics, small molecules, biologics, and medical countermeasures.

As NCI Director, I repeatedly spoke about how exciting a time it is to be a cancer researcher, because the pace of progress against cancer in recent years has been so remarkable.

Anyone who has been a part of that effort is aware of the key role that the FDA has played in this rapid progress against cancer.

So it is thrilling for me to come to the FDA and to work beyond cancer, to advance our public health mission across the entire spectrum of human and veterinary medicine.

One of the things I did in preparing for my new role was to make a point of speaking to several former FDA commissioners. I want to give a special word of thanks to Dr. Scott Gottlieb, who was extraordinarily supportive and helpful during the transition.

I received a lot of good advice from many different folks. And one of the most often-heard pieces was to "listen to the FDA experts around you."

I do plan to do that, to listen closely to FDA's great leadership across the Agency, as well as the talented workforce that offers such a remarkable range of skills.

And I would add that the "experts around me" include the strong and supportive community of stakeholders with which the FDA engages—including many of you here today—who are committed to our mission and who help ensure that we are informed and rigorous in executing our responsibilities.

A LANDSCAPE OF SUCCESS AND REFORM

It's in large part thanks to the hard work and commitment of our employees and stakeholders that the Agency's ability and reputation is stronger than ever—throughout the nation and in the halls of Congress.

It's a great time to be at the FDA. Advances in science are changing the trajectory of so many areas of public health.

And these developments offer new and novel ways to address and overcome major health challenges, from treatments and cures of deadly diseases to the ability to track, trace, and prevent dangerous outbreaks of food contamination.

FDA does a remarkable job applying the science that supports the development of these solutions.

I may be the Acting Commissioner, but I won't be acting as if this is a temporary job.

Secretary Azar and the White House made it very clear to me that they have been impressed with the things that FDA has been accomplishing and they don't want to disrupt this strong progress. There will be no pause at FDA.

So we'll continue to focus on innovation and efficiency, in order to bring more new medical products more quickly to the public, and to ensure that FDA's processes are as modern, efficient, and risk-based as possible across the Agency.

We want to continue to encourage the regulatory certainty, flexibility, and transparency that allows industry to innovate and the public and stakeholders to get what they need.

The evidence of our success is visible across the entire FDA landscape. Trying to capture even just the highlights in a speech like this is not possible.

But I'll give it a try . . . .

CENTER FOR DRUG EVALUATION AND RESEARCH

I'll start with the work of our medical product offices. Our expedited program tools—Fast Track, breakthrough, accelerated approval, and priority review—continue to help speed drug development and review.

Sponsors took advantage of at least one of these innovative expedited programs in seventy-three percent of the novel drug approvals last year by the Center for Drug Evaluation and Research (CDER).

And it was a record-setting year, with fifty-nine new novel drug approvals of products never before marketed in the U.S., as well as approvals of many others that have new and innovative uses.

The list of diseases these drugs are designed to treat is enormous, including the first drug ever to treat smallpox, a new single dose treatment for influenza, and a new class of drugs to treat patients with HIV-1 infection who have failed other therapies.

In cancer last year, the FDA approved new treatments for patients with breast cancer, prostate cancer, lung cancer, and thyroid cancer, as well as new therapies to treat blood cancers and other blood disorders.

As an oncologist, I used to treat acute leukemia, and for decades, we had no new therapies. But last year, FDA had eight new approvals for this disease. Eight!

It's important to reaffirm that as we work to make our process more efficient, we will always maintain our focus on the FDA's gold standard of safety and efficacy.

Safety is a priority before and after approval, and the public depends on our diligence in identifying issues when they occur.

This focus on safety drives our thinking about new products, too. For example, take CBD (or cannabidiol) products. As many of you probably know, these are made out of a non-psychoactive component of cannabis and are being marketed in a myriad of forms for humans (and pets). The regulation of a product like CBD is complex and touches almost every aspect of FDA's authority.

At the end of this month, we will be holding a public meeting on CBD where stakeholders can share their views on products that include it, to inform our regulatory path forward.

Once we've approved a new medicine, it's also important that the patients who need these groundbreaking treatments can get them.

To that end, we had a very strong year in our work to increase competition and to help reign in prescription drug costs through advances in our generic drug and biosimilars programs.

This past fiscal year we set an all-time record of 971 full and tentative approval actions for generic drugs, including ninety-five first-time generic drugs.

Importantly, this list includes several competitive generic therapy designations, developed to improve access and foster competition for drugs that face inadequate competition.

Likewise, many of the generic approvals were of complex generics, which can involve special challenges in "genericizing" and which may need additional support for their development.

Biological products play an increasingly critical role in treating many serious illnesses, including rare genetic disorders, autoimmune diseases, and cancer.

These complex products represent the fastest growing category of U.S. drug spending, accounting for seventy percent of the growth in drug spending from 2010 to 2015.

We launched our Biosimilars Action Plan to accelerate biosimilar competition.

This plan is beginning to work: last year, we approved a record number of biosimilar products, meaning patients can receive the treatments they need, while the market benefits from increased competition and ultimately lower costs.

We also have worked to limit shortages of medically necessary medications. I should note that shortages can occur not only for drugs, but for other types of products including devices and pharmaceuticals for animals.

Last year, we successfully worked with manufacturers to prevent 160 drug shortages and identified only fifty-four new shortages. That's about one fifth of the peak shortage year of 2011.

OPIOIDS

Although last year saw many FDA successes, we remain deeply aware of significant health challenges.

These include challenges in finding new treatments and cures for diseases, but also in addressing complex ongoing public health crises.

I'm referring most directly here to the continuing opioid crisis. This is one of the most troubling public health crises our nation has faced, causing widespread tragedy to families and undermining communities.

FDA has an important role to play in combatting the opioid crisis, both individually and in collaboration with other federal and state agencies.

We have implemented a multi-pronged strategy to address this complex challenge.

We're continuing our work to support the development of drugs to treat pain that are not addictive or are abuse-deterrent.

We are planning updated guidance outlining the appropriate clinical endpoints and clinical trial approaches for the development of non-opioid drugs for use in the treatment of acute and chronic pain.

And we'll also be exploring new methods for analyzing and evaluating abuse-deterrent features.

Congress's passage last year of the bipartisan Substance Use-Disorder Prevention that Promotes Opioid Recovery and Treatment for Patients and Communities Act (or the SUPPORT Act), authorized FDA to more forcefully address the opioid problem in several ways.

To give you just one example, the law allows FDA to require certain packaging configurations, including short-duration packaging for outpatient dispensing. Our

research suggests that for many acute pain indications where opioids are used, just a day or two of these drugs is appropriate.

We're considering whether to mandate that certain oral forms of immediate-release opioid formulations be made available in small quantities in blister packaging. You'll be hearing more about this in the near future.

An area I find personally exciting is our new enforcement activities and authorities targeting those who unlawfully market or distribute illicit opioids and other unapproved drugs.

Our Office of Regulatory Affairs (ORA) and CDER have already made significant progress stopping illegal activities at the border.

We've expanded the capacity of import operations, made significant investments in our Office of Criminal Investigations, and our laboratories, including our Forensic Chemistry Center.

We have a long way to go to defeat this tragedy. But we are making headway, and it remains one of our highest priorities.

CENTER FOR DEVICES AND RADIOLOGICAL HEALTH

When it comes to applying new science and technology to the development of promising devices and diagnostics, the Center for Devices and Radiological Health is leading the way.

There are more than 190,000 regulated medical devices, produced by 18,000 device manufacturing firms, in 21,000 manufacturing facilities across the globe.

And the range of products and their level of sophistication is just as diverse, including everything from tongue depressors, to tissue implants, to next generation sequencing.

Last year, our medical device program approved 106 novel devices, surpassing the forty-year record FDA set . . . the year before, and continuing on a steady upward climb.

And these are not cookie-cutter approvals. Indeed, looking at the technological and scientific sophistication of some of these products, you might think you're watching a sci-fi movie.

To ensure that this progress continues, last year the Center released its Medical Device Safety Action Plan, which charts a course for how FDA will maintain and strengthen its ability to advance medical device safety and innovation in the coming years.

It builds on enhancements to our postmarket surveillance, 510(k) and De Novo review processes, and includes a final guidance on our successful Breakthrough Device Program and plans to establish our new Safer Technologies Program (or STeP).

This year we also launched the Digital Health Pre-Cert Pilot program, an innovative, streamlined approach to helping software developers get safe and effective digital health products to providers and patients more quickly.

When it comes to embracing state-of-the art science, the work being done on the In Vitro clinical tests may set the standard.

We support a legislative solution that presents a consistent approach for all IVCTs, regardless of whether the test developer is a conventional manufacturer or a clinical laboratory, and we are committed to making this an Agency priority.

CENTER FOR BIOLOGICS EVALUATION AND RESEARCH

I've already mentioned biologics and biosimilars, but I wanted to spend more time highlighting the groundbreaking work in our Center for Biologics Evaluation and Research involving regulation of the development of safe blood products, innovative cellular therapies, and other complex biological products, including gene therapies.

It's been a very busy period. The past year saw the approval of new blood products, as well as approvals of important tests for use in screening the blood supply.

And lots of progress on vaccines, including a first ever vaccine to prevent Dengue Fever announced just yesterday.

Two areas in this Center have seen particularly large attention and growth.

The first is gene therapies. This new era of medicine has arrived.

We received over 200 gene therapy INDs last year. Our goal is to support this work and advance the field while being transparent about our expectations. As a result, we issued numerous guidance documents with more on the way.

The second area of CBER where we're seeing an extraordinary pace of progress is in the development of cellular therapies and, especially, stem cell products. We're doing everything we can to support this growing industry. But even as we do so, we want to maintain our focus on compliance and enforcement.

We won't tolerate dangerous stem cell clinics who make dubious or unproven claims that their cellular products will treat age-related or other serious or life-threatening conditions.

Before I move on from CBER, let me comment on our continued emphasis on communicating about the safety and effectiveness (and availability) of vaccines to prevent infectious disease like influenza, HPV, and measles.

It's disheartening to see the current outbreak of measles, a serious life-threatening infection that we had eliminated from the U.S., now making a tragic comeback because of vaccine avoidance.

FOODS AND NUTRITION

One area of FDA's responsibilities that I'm especially enthusiastic to take on is the work the Agency does in nutrition and food safety.

The work in food is wide-ranging, from genome sequencing of pathogens to animal cell-culture technology; from inspections of produce and imported food to gene editing.

Let me start with nutrition. As a physician, I have long appreciated the profound impact that improvements in diet and nutrition can have on human health through the reduction of many chronic diseases, from cancer to diabetes to heart disease.

Consider this statistic from the American Heart Association—more than twenty percent of all deaths in the U.S. in 2015 were attributable in part to a poor diet.

FDA does significant work in strengthening nutrition and working to improve the public's health, through our efforts on labeling for example, along with actions we have taken with regard to trans-fatty acids.

We're building on this important work with our comprehensive, multi-year Nutrition Innovation Strategy, announced this past year.

This strategy encompasses two important goals: first, to reduce preventable death and disease caused by poor nutrition by ensuring that consumers have access to

accurate information to support healthy food choices and, second, to foster the development of healthier foods.

Few areas of FDA's work affect as many Americans on a daily basis as what we do to ensure food safety and prevent foodborne illness. A centerpiece of our work here continues to be the implementation of the landmark FSMA law.

We know that many stakeholders, such as farmers and importers, are experiencing a new level of oversight, and we will continue to ensure they have the resources they need to comply with these important regulations.

For example, we've trained thousands of farmers on the requirements of the Produce Safety Rule through our various collaborations.

When it comes to the safety of imported foods, we're working to integrate our new import oversight tools with existing tools.

Earlier this year, we released a new Strategy for the Safety of Imported Foods, which highlights how FDA is using new tools like the Foreign Supplier Verification Programs to enhance our oversight of imported foods.

Today, we're doing even more to build on the vision of FSMA through an exciting new focus just announced by Deputy Commissioner Frank Yiannas and myself—a strategy for a new era of smarter food safety.

To kick this off we are developing a "Blueprint for a New Era of Smarter Food Safety," and will be holding a public meeting later this year to seek stakeholder input.

The Blueprint will allow us to upgrade our ability to rapidly track and trace food through the supply chain—making it possible to find the origin of contaminated food in a matter of minutes, rather than days or weeks.

As part of this work we also plan to conduct a new pilot using artificial intelligence to enhance the Agency's review of imported foods at ports of entry into the U.S.

The number of import food lines is increasing every year, and applying the best predictive and analytical tools will help ensure we're targeting the greatest risks to protect consumers.

CENTER FOR VETERINARY MEDICINE

The Center for Veterinary Medicine is a microcosm of the FDA, regulating a fascinating portfolio of technological innovation.

As an example, intentional genomic alterations (or IGAs) in animals have the potential to improve nutrition, assist farmers in addressing animal health issues, produce substances for novel drugs, and yield tissues and organs for xenotransplantation.

With regard to intentional genomic alteration, CVM's role is to make sure that the genetic modification is safe to the animal, that it does what it's supposed to do, and, if in a food-producing animal, that it is safe to eat.

That's why we have expanded our scientific expertise and hired additional staff to support our science- and risk-based evaluation of these products.

We encourage developers to interact with us early and on a regular basis as they move forward with these products.

We intend to release additional guidance in the coming year to provide more clarity about our risk-based framework and policy of enforcement discretion pertaining to the regulation of IGA animals.

Additionally, CVM has adopted a "One Health" approach to addressing public health challenges with respect to animal drugs and animal food, helping to address both human and animal health concerns.

The Center is also fostering innovation of new animal drugs with implementation of its new authority for expanded conditional approval to address certain serious or life-threatening conditions, or unmet health needs.

CVM's scientists are also at the forefront of using new models to reduce, replace, and refine the use of animals in research through an innovative bioequivalence study.

COSMETICS

The FDA regulates cosmetics, sometimes people forget. That's somewhat understandable, since when it comes to overseeing the multi-billion-dollar cosmetics industry, the FDA faces some limitations under the law.

The FD&C Act doesn't require that cosmetics or their ingredients be reviewed or approved by FDA prior to sale, except color additives.

But that doesn't mean we don't take action when a public health hazard is identified. For example, we are working to protect consumers from tattoo inks that have microbial contamination.

And we also have responded to concerns over the possibility of asbestos contamination of talc in some cosmetics. The latter concern led to an FDA warning to consumers earlier this year.

If Congress sees fit to change the current regulatory paradigm for cosmetics, there are a number of areas of focus it could look at, including mandatory registration and listing, good manufacturing practice regulations, mandatory reporting of adverse events, access to records, mandatory recall, and the labeling of known allergens.

We look forward to informing and supporting the efforts of Congress and stakeholders, should there be interest in exploring changes in the law.

DIETARY SUPPLEMENTS

Another priority for FDA involves dietary supplements: our work to modernize and reform our oversight and strengthen our enforcement strategies in this area.

FDA's Office of Dietary Supplement Programs has grown since it was created in late 2015, and today has nearly two dozen employees.

But this office faces enormous challenges in regulating an industry that has grown tenfold over the last twenty-five years, since the Dietary Supplement Health and Education Act was enacted.

The Dietary Supplement industry now has estimated annual sales exceeding $40 billion, and a market that has grown from about 4,000 products to as many as 80,000.

What is more, as supplements have grown in popularity, so have the number of products on the market that are potentially dangerous, or that make misleading health benefit claims.

As an oncologist, I've seen first-hand how charlatans will sell false hope to prey on desperate patients with cancer and other diseases.

Even as the FDA works toward modernizing our oversight structure, we'll continue to protect consumers by cracking down on false, misleading, and potentially harmful claims by some of these manufacturers.

Next month we will be holding a Public Meeting on supplements, and I look forward to beginning a productive dialogue about reshaping our oversight of these products

OFFICE OF REGULATORY AFFAIRS

Throughout my remarks today, I have alluded to the important role that FDA's investigation and enforcement efforts have played in our broader work.

This underscores the critical role of the Office of Regulatory Affairs (or ORA), the heartbeat of FDA if you will, in everything we do across the Agency.

ORA's vital staff, spread out across the nation and the world, make sure that FDA's words have meaning, and that its actions have teeth.

So let me offer a special recognition to the men and women of ORA, who play such a pivotal role working from our field offices, laboratories, and other locations across the globe.

For instance, they are working on fully implementing the very important Pharmaceutical Annex to the Mutual Recognition Agreement with the European Union, which will improve FDA's global oversight of pharmaceutical manufacturing.

Under this agreement, the U.S. and EU regulators can utilize each other's good manufacturing practice inspections of pharmaceutical manufacturing facilities.

We expect to complete all the remaining assessments in the next several months, on schedule.

And the Pharma program is working with CVM to prepare for its own EU assessment of FDA's oversight of animal drugs.

CENTER FOR TOBACCO PRODUCTS

Finally, our Center for Tobacco Products (or CTP) has been working to end the addiction to dangerous tobacco products among adults, and on preventing young people from starting.

Over the last year we've taken a number of actions, including three important advance notices of proposed rulemaking: on Tobacco Product Standard for Nicotine Level of Combusted Cigarettes, on Regulation of Flavors in Tobacco Products, and on the Regulation of Premium Cigars.

CTP has been focused on acquiring further data on electronic cigarette products, so that we can make informed regulatory decisions to reverse a growing epidemic of use of Electronic Nicotine Delivery Systems (or ENDS) by children.

We issued a Youth Tobacco Prevention Plan and expanded public education efforts to prevent youth use of ENDS. And we remain committed to protecting youth from other tobacco products.

We ramped up our enforcement efforts, issuing warning letters, civil money penalties, and no-tobacco sale order to retail establishments selling to minors, particularly those selling flavored e-cigarettes, and to companies selling e-liquids with labeling or advertising that resemble kid-friendly food products.

The bottom line is, we won't tolerate misleading marketing or the selling of tobacco products to children.

LOOKING FORWARD—NEW CHALLENGES

Whew! That's a big portfolio. And that summary barely scratches the surface.

Hopefully, now you can see now why I'm never bored at work.

To conclude, as a biomedical researcher, I learned that disease doesn't respect or recognize the silos we've created between basic and applied research, between clinical care and drug development, or between the disparate systems providers use to guide patient care.

If we want to harness the full potential of therapies to transform care, we need to become more efficient, more collaborative, and more data-driven so that we can learn from every patient's journey.

We need to do that across FDA, by advancing recent institutional, structural, and policy changes that allow the Agency to more effectively apply science in support of innovation while ensuring protection of the public.

But we also need better collaboration. FDA has a long history of successful collaboration with a wide variety of medical and scientific organizations, patients and their advocacy groups, and, yes, even legal organizations. And we also collaborate across government with other agencies.

We need to ask ourselves if the FDA organizational structure is maximally efficient, and one that integrates people from different disciplines and across different states of a product's lifecycle.

To fully and effectively achieve FDA's mission to promote and protect public health, we must seize the opportunities offered by the scientific and technological advances.

It is crucial that FDA be nimble, flexible, and adaptable as we continue to advance regulatory frameworks, products, and interventions.

The pace of innovation is incredible. There's been no other time in history where scientists and researchers discovered so many novel therapies and been on the cusp of so many scientific breakthroughs.

What is not lost on me is that no matter the discovery, FDA will be key to bringing new science to those who need it.

I'm honored to be part of such an amazing institution and community. I look forward to what lies ahead as we work together to meet our shared public health goals.

Thank you.