

# FDA in the Post-Gottlieb Era

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May 22, 2019

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Coalition for Healthcare Communication (CHC):  
Rising Leaders Conference on Healthcare Policy



Nancy Bradish Myers, Esq.

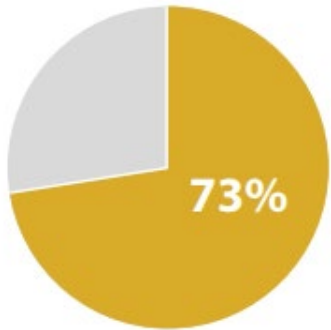
# Overview

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- Gottlieb: What did we like?
- Sharpless' FDA: What we know and what to watch for
- Potential icebergs that could impact FDA progress
- Initiatives to watch
- Conclusions

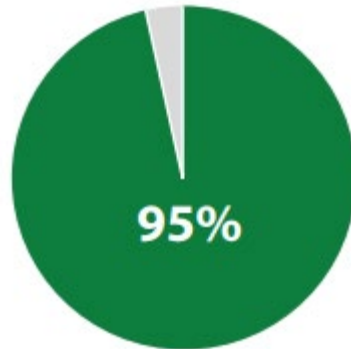
# What Did We Like About Gottlieb as Commissioner?

**43 of the 59** novel drug approvals of 2018 (73%) were designated in one or more expedited categories of Fast Track, Breakthrough, Priority Review, and/or Accelerated Approval



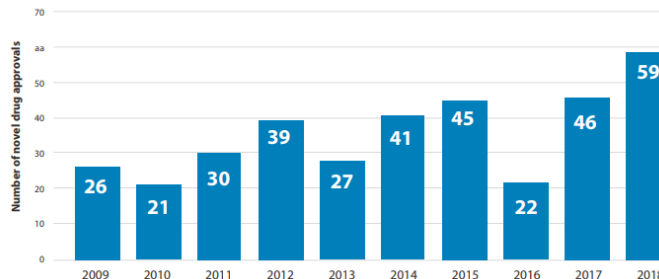
Source: CDER 2018 New Drug Therapy Approvals report

CDER approved **56 of the 59** novel drugs of 2018 (95%) on the “first cycle” of review



## CDER's Annual Novel Drug Approvals: 2009 - 2018

In 2018, CDER approved 59 novel drugs. The 10-year graph below shows that from 2009 through 2017, CDER has averaged about 33 novel drug approvals per year.



## THE NUMBERS!

### Policy by tweet:

- Averaged over 14 tweets per day in 2018
- Set a personal record of 554 total tweets in one month (July 2018)

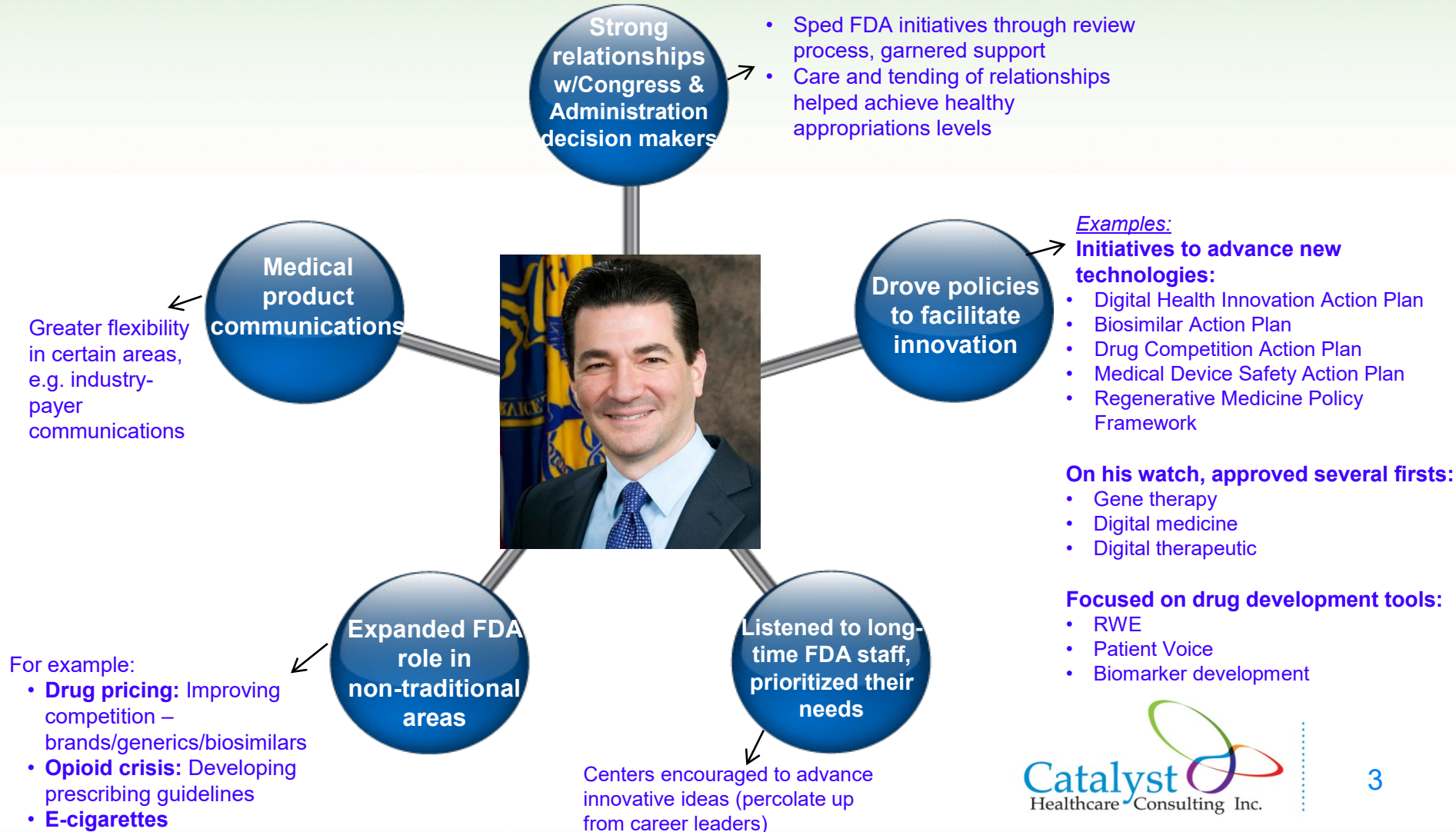
Source: Biopharma Dive [article](#), Aug. 27, 2018

### Rapid pace of guidances:

- Total of 414 guidances issued during Gottlieb's tenure (May 11, 2017 – April 5, 2019)

Sources: [Politico](#), "The Last Day of Commissioner Gottlieb," 4/1/19; FDA [guidance page](#).

# Gottlieb: Why the Rave Reviews?





# Acting Commissioner Sharpless: What We Know About His Agenda

## Early messages:

- **Working to maintain Administration support**
- **Plans to continue with current priorities and drive existing action plans:**
  - Focus on innovation and efficacy
    - Continued focus on use of expedited pathways
    - Improving efficiency of new product reviews
    - New guidance/transparency for development of gene & cell therapies
    - Continued implementation of the Biosimilars Action Plan
  - Focus on safety
- **Recognition that internal experts know something**

“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.”



Acting Commissioner Sharpless, FDLI annual meeting, May 2, 2019

# Acting Commissioner Sharpless: Personal Priorities Prior to FDA

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- Before coming to FDA, Sharpless' stated certain areas of interest, including:
  - **Modernizing clinical trials**
    - Modernizing infrastructure & improving efficiency
  - **Use of big data**, including the need to:
    - Move away from passive data aggregation
    - Link complex data sets
    - Understand cancer care and generate RWE
  - **Importance and role of biomarkers**
    - e.g, to predict toxicity from surgery or chemotherapy
  - **Reducing smoking rates & youth vaping**
    - Expressed support for initiatives such as:
      - FDA's youth e-cigarette prevention campaign
      - American Cancer Society's annual Great American Smokeout

# Environmental Icebergs to Watch For



OMB Policies

Politics leading  
into 2020  
elections

Global trade  
issues

Focus on  
safety

Drug  
Importation

# Potential Icebergs: New OMB Policy May Slow or Politicize Guidance Development

- **OMB recently issued a memorandum\* reaffirming its authority to require prior review of all rules - including guidances**
  - Includes new process for how OMB will determine whether a proposed rule/guidance qualifies as “major”
  - Agencies must submit “major” rules/guidance to Congress for prior review
- **Concern:**
  - FDA cannot publicly vocalize concerns as part of the Administration
  - Media waiting for a situation where a guidance is held up
- **If OMB applies this to FDA in a stringent way, it could:**
  - **Slow FDA’s efforts to advance new policies and advice quickly**
  - 2019 is a big guidance year:
    - CDER – expects to issue 93 new guidances
    - CBER – expects to issue 19 new guidances

\* OMB memorandum M-19-1, “Guidance on Compliance with Congressional Review Act”



# Potential Iceberg: 2020 Elections and Contentious Political Environment

- **FDA is a science-based organization, but the 2020 elections could impact decisions like:**
  - **Deciding not to nominate and confirm a Commissioner**
    - If Trump doesn't want to spend political capital to nominate and convince Senate to confirm, this would leave Sharpless as Acting, in a caretaker role
  - **Prescription drug prices and health policy that could impact FDA authorities:**
    - Importation
  - **Trade war issues**
    - Relationships with foreign regulators: e.g., China
- **Presidential election:**
  - Any unforeseen crisis could give Democratic Presidential contenders fodder for criticizing the Administration and scrutinizing FDA, e.g. -
    - Any new widespread food-borne illness/outbreak
    - Any new patient safety issue
- **House Democrats' investigations**

# Potential Iceberg: Importation Gaining Steam as Means to Address High Drug Pricing

## Latest Effort: FL importation bill, Gov. DeSantis and Trump support:

- Authorizes FL to import drugs:
  - **From Canada** – for Medicaid, corrections, other state programs
    - Requires compliance with US track & trace requirements/Drug Quality & Security Act
    - Vendor must certify imported drugs not adulterated or misbranded
  - **More broadly/internationally**– for all other FL residents
    - Requires “export pharmacy” inspection reports, either from country of origin, FDA or state of FL
    - Limited to countries that have mutual recognition agreement/MOU/other Good Manufacturing Practice policy
  - Trump has directed Sec. Azar to work with FL to get the plan approved
  - FL must submit a plan for approval by mid-2020 - but Trump favors quicker action
- **Social media campaigns ramping up:**
  - “Caravans to Canada” – parents seeking to buy cheaper insulin for their kids
  - Change.org petitions
- **Many states calling for federal government to take the lead**
- **Counterfeit concerns falling of deaf ears**

## What does this all mean for FDA?

- Pressure on Azar to require FDA to take steps to permit this
- Any safety crisis linked to imported drugs could put FDA in the crosshairs
- It will complicate post marketing surveillance and AE tracking in the US

# Potential Iceberg: Global Politics and Trade Issues

- **Few discussing this yet, but a trade war with China could indirectly impact FDA**
  - Active pharmaceutical ingredients (APIs): Much is sourced ex-US
    - **~80% of the ingredients** in U.S.-branded pharmaceuticals and OTC drugs are sourced from either **China or India\***
  - A prolonged trade war with China could result in higher prices and/or potential shortages



\*Source: NBC News, Aug. 14, 2018, citing "China Rx: Exposing the Risks of America's Dependence on China for Medicine," by Rosemary Gibson and Janardan Prasad Singh

# Potential Iceberg: Focus on Safety

## Consumer groups continue to be active in this area:

- **Public Citizen** recently brought suit against FDA for failure to act on a 2016 petition re: Parkinson's drugs and compulsive/uncontrollable behaviors
  - Behaviors related to 6 dopamine agonists used to treat Parkinson's, restless leg syndrome
  - **Lawsuit seeks to force FDA to require:**
    - Black box warnings
    - Letters to health care providers, patients
- **Public Citizen also recently petitioned FDA for:**
  - Black box warning for Amgen's Prolia, for osteoporosis, on risk of vertebral fractures after stopping the drug (April 2019)
  - Moratorium on approval of new opioids or opioid formulations (April 2019)

## Examples of recent steps FDA has taken on safety:

- **Urogynecologic surgical mesh implants**
  - FDA ordered companies to "stop selling and distributing" products immediately due to lack of demonstrating reasonable assurance of safety/effectiveness (April 2019)
  - Public Citizen recently urged a ban (Feb. FDA meeting) and earlier petitioned for a ban (2011)
- **Takeda's Uloric for treatment of gout**
  - FDA now requiring boxed warning on increased risk of death vs. another gout drug (Feb. 2019)
  - In 2018 Public Citizen urged FDA to ban Uloric due to safety risks, lack of benefit

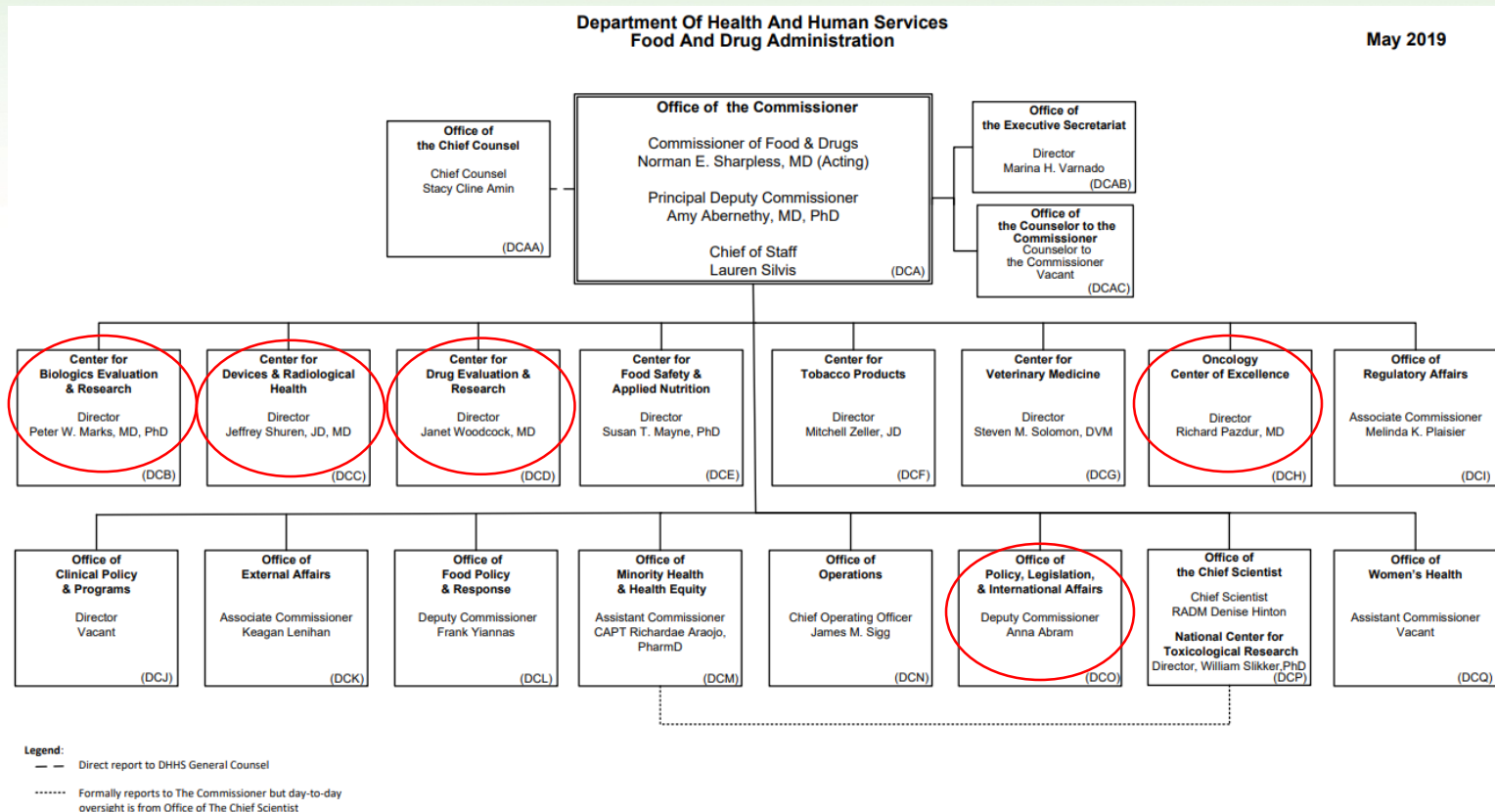
# 2019: What Interesting Issues Will be on FDA's Plate?

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# Major Initiatives: Implementing Organizational Changes – OC



- **Sharpless inherits a more streamlined, tightly organized Office of the Commissioner with a large number of direct reports including Center Directors**

# Major Initiatives: Implementing Organizational Changes - CDER

## Implementation of CDER reorg under way

- Intended to improve efficiency, accommodate growing volume and complexity of workload

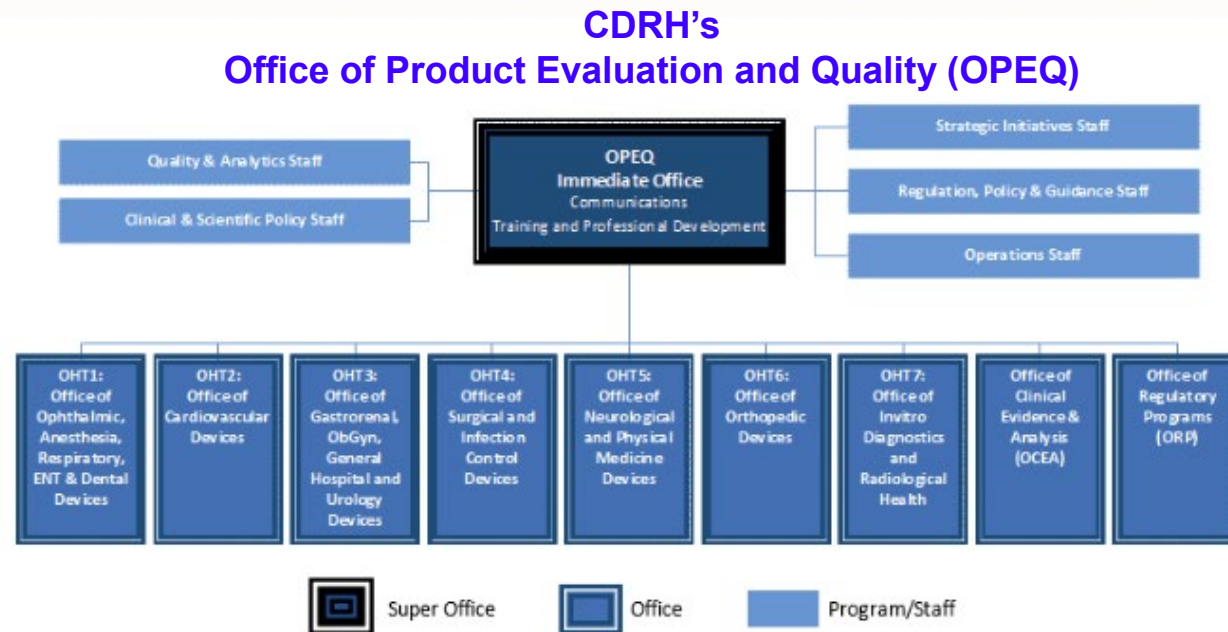
### Key elements:

- **Office of New Drugs (OND) reorg**
  - Flattening out of Offices/Review Divisions
  - More logical groupings of diseases
  - **Part of broader modernization of OND's operations**
- **Office of Drug Evaluation Science (ODES):**
  - **New office:** Charged with creating structured, standardized approaches to data evaluation
  - Intended to support transition to a **revamped drug review process**
- Office of Therapeutic Biologics and Biosimilars (OTBB):
  - **Dedicated Biosimilars office** intended to strengthen biosimilar product review, advance policy development

# Major Initiatives: Implementing Organizational Changes - CDRH

**CDRH reorg under way** to improve efficiency, create more team-based approach to regulation

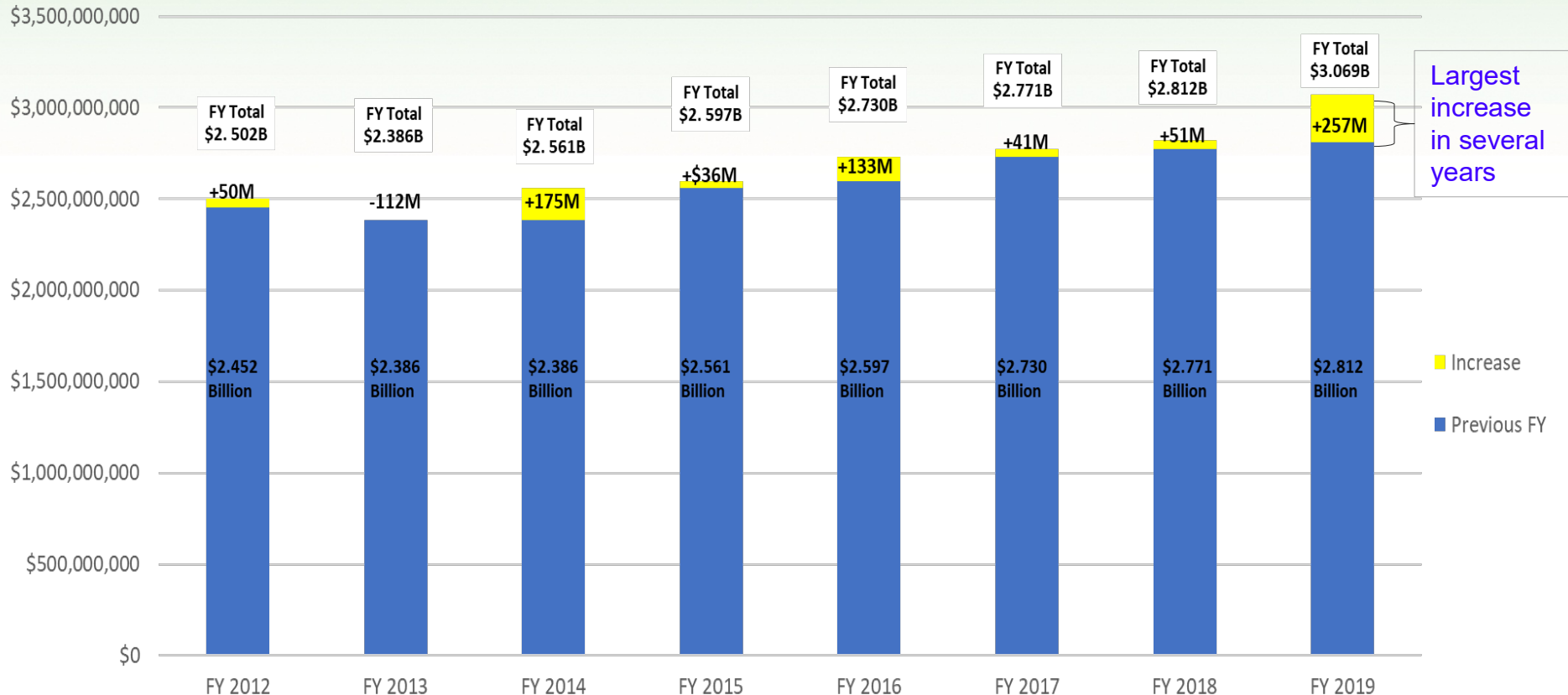
- Establishes new **Office of Product Evaluation & Quality (OPEQ)** “**Super Office**” (as of May 1)
- Integrates product teams across the Total Product Life Cycle (premarket through post-market phases)



Source: FDA web site,  
as of 5/1/19

**Safety was part of rationale for the reorg:** specifically, that safety issues may not surface until the post-market phase.

# Major Initiatives: Appropriations - Current & Future Funding Levels



\*FDA Budget Sequestered

\*21<sup>st</sup> Century Cures Adds to Total Through FY25

Source: Alliance for a Stronger FDA

# Major Initiatives: Appropriations - Current & Future Funding Levels



Current FY 2019 appropriations:	President's FY 2020 budget request
FDA received a <b>\$257 million increase</b> over prior year, for total appropriations of <b>\$3.069 billion</b>  This is the largest increase in a number of years	FDA would receive a <b>\$350 million increase</b> in overall budget vs. prior year

Source: Alliance for a Stronger FDA

## New initiatives supported by FY 2020 budget request include:

- **Advancing modern manufacturing technologies** for drugs/biologics
- **Advancing use of RWE to create near-real-time data evaluation capability**
  - Would use machine learning and cover gaps in Sentinel & NEST to evaluate safety & effectiveness of marketed products
- **Facilitating Rare Disease drug development** - Developing clinical trial networks to understand natural history & clinical outcomes of RDs
- **Addressing opioids** – e.g., implementing SUPPORT Act
- **Improving IT infrastructure** to support regulatory review and knowledge management

Source: President's FY 2020 budget request to Congress



# Major Initiatives: Regulation of Promotional Messages

- **FDA is considering/developing guidance in several areas** to modernize its approach to regulation of labeling, advertising, promotion & other medical product communications, e.g.-
  - **Scientific exchange**
  - **Labeling considerations to reduce medication errors**
    - “Serious public health concern for the agency”
- **Biosimilars:**
  - FDA will take new actions to promote appropriate adoption of biosimilars, “stop any false or misleading promotion”
- **FDA has recently issued these guidances (2018):**
  - **Health care economic information** (manufacturers to payors)
  - **Communications “consistent with FDA-required labeling”** (off-label)
  - **Quantitative efficacy and risk information in DTC ads**

## **Drug pricing & DTC ads:**

- HHS recently issued a final rule to use DTC ads as vehicle to inform consumers re: drug costs
- Requires DTC ads for drugs reimbursed by Medicare or Medicaid to include information on list price (WAC)
- Effective date: July 9, 2019

# Major Initiatives: Modernizing Clinical Trials/ Improving Trial Design

**Sharpless** and his top deputy/Acting CIO **Amy Abernethy** plan to build on FDA's existing efforts to modernize clinical trials & data generation

**Sharpless'** areas of focus will likely include:

- **Use of RWE as a means to speed clinical trials**
- **Modernizing trial infrastructure & improving efficiency**

**Abernethy is strongly focused on modernizing/maximizing data generation**, eg –

- Aggregating data across settings, to improve both clinical care & data generation
- Using CDRH's Total Product Life Cycle (TPLC) approach as a model - “prototype for the future”

“To unleash the full potential of a learning health care system – to deliver improved patient outcomes at a sustainable cost without sacrificing safety – the FDA is going to have to learn how to apply and scale” strategies similar to CDRH's TPLC approach “across its entire regulatory portfolio. In fact, I'd go so far as to say we don't have a choice.”– Amy Abernethy, MDMA annual meeting, May 2, 2019

# Major Initiatives: Accelerating Product Approvals

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- In initial speeches as Acting Commissioner, Sharpless continues to advance the concept of efficient, flexible regulation, in order to advance new products
- However, thus far, he has not offered specifics in terms of policy actions he plans to take

# Next Permanent Commissioner: Qualifications



## Preferred candidate - Qualifications:

- ✓ Visionary, decisive leader
- ✓ Strong scientific/technical expertise
- ✓ Strong communicator, internally & externally
- ✓ Public health focus
- ✓ Commitment to facilitating innovation
- ✓ Strong advocate for FDA mission, staff & appropriations
- ✓ Support for career leadership at FDA
- ✓ Politically astute
  - Able to foster relationships with Congress, industry, patient groups & others
  - Able to position FDA well within HHS, White House

# Conclusions

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- 2019 could be a year of surprises, but most are hoping for a year of continuing implementation of action plans created under Gottlieb:
  - Delivering on legislative requirements
  - Delivering on user fee-negotiated milestones (and preparing/aligning for next user fee cycle)
  - Continuation of action plans

However, surprises pop up periodically and could come from:

- Political dynamics
- Uncertain global events
- Staff changes
- and ...



# Questions?

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Contact information:  
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Catalyst Healthcare Consulting, Inc.  
7704 Georgetown Pike  
McLean, VA 22102

703-448-7448  
Nancy@catalysthcc.com



Nancy Bradish Myers, Esq.