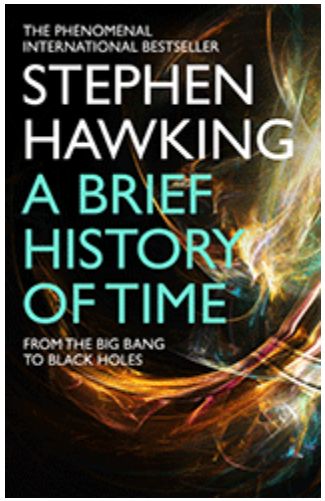
Opening Address

Jeffrey M. Bockman, PhD
Vice President
Defined Health

CANCER PROGRESS
by Defined Health
March 8 - 9, 2016
New York, NY

A Brief History of Time*



23rd ANNUAL
CANCER PROGRESS
CONFERENCE
March 6 & 7, 2012

*Well, At Least Defined Health's Time With Cancer Progress

Cancer Progress Parallels The Changing Oncology Landscape

2012

- Biologics and Small Molecule Inhibitors Driving Growth Producing More Potential Blockbuster Orphan/Niche Products
- Angiogenesis and Tumor Microenvironment Targets Remain Significant
- Immuno-Oncology Sparking Some Interest

2013

2014

2015

2016

Cancer Progress Parallels The Changing Oncology Landscape

2012

2013

2014

2015

2016

23rd ANNUAL CANCER PROGRESS CONFERENCE

Keynote Addresses

- **Angiogenesis:**
  - **George D. Yancopoulos, MD, PhD**
- **Hematology State of Art:**
  - **Kenneth C. Anderson, MD**
- **Mark McClellan, MD, PhD**

Selected Panel Topics

- Immunotherapy for cancer – Are we there yet?
- Melanoma: Tackling the intractable
- Novel MOAs and targets for cancer drug development
- Comparative effectiveness and reimbursement – Are we setting the bar sufficiently high?
- Biomarkers, companion diagnostics and enabling technologies and services for personalized medicine

Selected Moderators & Panelists

- Neal Rosen, MD
  - Memorial Sloan-Kettering
- Steven Averbuch, MD
  - Bristol-Myers Squibb
- Richard Gaynor, MD
  - Eli Lilly
- Eric Rowinsky, MD
  - Stemline Therapeutics
- Jedd Wolchok, MD, PhD
  - Memorial Sloan-Kettering
- Jon Wiggington, MD
  - Bristol-Myers Squibb
- Brian Leyland-Jones, MD, PhD
  - Emory University
- John C. Reed, MD, PhD
  - Sanford-Burnham Medical Institute
- Philip W. Kantoff, MD
  - Dana-Farber Cancer Institute
- Robert C. Bast, MD
  - MD Anderson
- Robert Schneider, PhD
  - New York University

Cancer Progress by Defined Health
New York, NY | March 8-9, 2016

DefinedHealth unconventional insight

THERAPEUTIC INSIGHT by Defined Health

CANCER PROGRESS by Defined Health

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2014

2015

2016

Cancer Progress Parallels The Changing Oncology Landscape

2012

2013

2014

2015

2016

24th ANNUAL CANCER PROGRESS CONFERENCE

Keynote Addresses

- **The Evolving Landscape of Oncology Cost Containment:**
  - **Peter Bach, MD**
- **Giulio Draetta, PhD, MD**

Selected Panel Topics

- Next Generation Diagnostic Approaches
- Cancer Metabolism
- Cancer Stem Cells: Getting Past the Controversies
- Epigenetics – Are We There Yet?
- Immunotherapy, Bringing It All Together: The Next Generation of Approaches & Combos
- Next Generation Biologics: Beyond the Success of Conventional Mabs

Selected Moderators & Panelists

- Chris H. Takimoto, MD, PhD
  - Janssen
- Nicholas J. Sarlis, MD, PhD
  - Incyte
- Michael Pellini, MD
  - Foundation Medicine
- Neil Berinstein
  - Sunnybrook Research Institute
- Michael M. Morrissey, PhD
  - Exelixis
- Axel Hoos, MD
  - GlaxoSmithKline
- George Q. Daley
  - Dana –Farber Cancer Institute
- Christoph Westphal, MD, PhD
  - Verastem
- Stephen B. Baylin, MD
  - Sidney Kimmel Cancer Center
- Jordan D. Berlin, MD
  - Vanderbilt-Ingram Cancer Center
- Charles J. Link, Jr, MD
  - NewLink Genetics

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Cancer Progress Parallels The Changing Oncology Landscape

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2015

2016

CANCER PROGRESS by Defined Health
March 4 - 5, 2014
New York, NY

Keynote Addresses

- **The State of Play in Immuno-Oncology:**
  - **Axel Hoos, MD**
- **Valuing Value in Oncology:**
  - **Peter Bach, MD**
  - **David Haslam, FRCGP, FRCP**
  - **Clifford Hudis, MD**

Selected Panel Topics

- Taking Immunotherapy Seriously
- Big Data and Cancer
- ADC: Supercharge the Blockbuster Ab Class
- Cancer Gene & Cell Therapy
- Intrinsic and Acquired Resistance in Cancer
- Translational Oncology: How to Better Transform Targets into Relevant Drugs for Patients

Selected Moderators & Panelists

- Ira Mellman, PhD
  - Genentech
- Michel Sadelain, MD, PhD
  - Memorial Sloan Kettering
- Thomas Davis, MD
  - Celldex Therapeutics
- Jeffrey A. Engelman, MD, PhD
  - Massachusetts General
- Giulio Draetta, MD,PhD
  - MD Anderson
- Michael Kolodziej, MD
  - Aetna
- Richard Gaynor, MD
  - Eli Lilly
- Neal Rosen, MD, PhD
  - Memorial Sloan Kettering
- Philip Kantoff, MD
  - Dana-Farber Cancer Institute
- Robert Cohen, MD
  - Calico Life Sciences
- Renier J. Brentjens, MD, PhD
  - Memorial Sloan Kettering

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- **Targeted Therapies May Not Be Enough Alone But Will Remain a Major Part of Oncology Market**
- **Pricing Becomes a Talking Point and is a Future Concern**

2016

Cancer Progress Parallels The Changing Oncology Landscape

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2014

2015

2016

CANCER PROGRESS by Defined Health
March 17 - 18, 2015
New York, NY

Keynote Addresses

- **Technology and Big Data: Transforming Cancer Research and Care:**
  - **Lydia Chin, MD**
- **Progress at What Price?:**
  - **Peter Bach, MD;**
  - **Michael Kolodziej, MD**
  - **Lee Newcomer, MD**
  - **Leonard Saltz, MD**

Selected Panel Topics

- Targeting Checkpoint, Co-Stimulatory, and Novel Immunomodulatory MOAs
- Engineered Cell Therapy
- Combination Therapies: Challenges & Opps
- Bucket Trials and Drug Umbrellas
- Moving Beyond Mutations to Key Drivers

Selected Moderators & Panelists

- Otis Webb Brawley, MD, FACP
  - American Cancer Society
- Manfred Lehnert, MD
  - Takeda Pharmaceuticals
- Louis J. DeGennaro
  - Leukemia & Lymphoma Society
- Peter Sandor, MD, MBA
  - Amgen
- Axel Hoos, MD, PhD
  - GlaxoSmithKline
- Jedd Wolchok, MD, PhD
  - Memorial Sloan Kettering
- Thomas J. Kipps, MD, PhD
  - UC San Diego Moores Cancer Ctr
- Thomas O. Daniel, MD
  - Celgene
- Marc Theoret, MD
  - FDA
- Robert Cohen, MD
  - Calico Life Sciences
- Neal Rosen, MD
  - Memorial Sloan Kettering

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Cancer Progress Parallels The Changing Oncology Landscape

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2016

CANCER PROGRESS by Defined Health
March 8 - 9, 2016
New York, NY

Keynote Addresses

- **Cancer Moonshot 2020 & NIC:**
  - **Patrick Soon-Shiong, MD**
- **Game Theory of the Global Cancer Moonshoot:**
  - **Peter Kolchinsky, PhD**

Guest Moderators

- Paul B. Chapman, MD
  - Memorial Sloan Kettering
- Mark Simon
  - Torreya Partners
- Jeremy P. Goldberg
  - Torreya Partners
- Brian Leyland-Jones, MBBS, PhD
  - Avera Cancer Institute

Day 1 Panel Topics

- Revisiting Melanoma: From Intractable to Curable
- Myeloma Opportunities and Challenges in Moving Towards a Cure
- Investing in Oncology – A New Math or Same Old Same Old?
- From Novel Science to Clinical Development: Stories from Small Biotechs
- Parsing the Data, Vetting the Value

Day 2 Panel Topics

- Immuno-Oncology I: Attacking Cancer Antigens – Personalized Vaccines & Triggering of Immunogenic Cell Death
- Immuno-Oncology II: Next Wave IO Targets and Modalities
- N-of-One Trials: Is This the Future of Oncology Drug Development?
- Novel MOA: RNA as a Target and a Therapeutic

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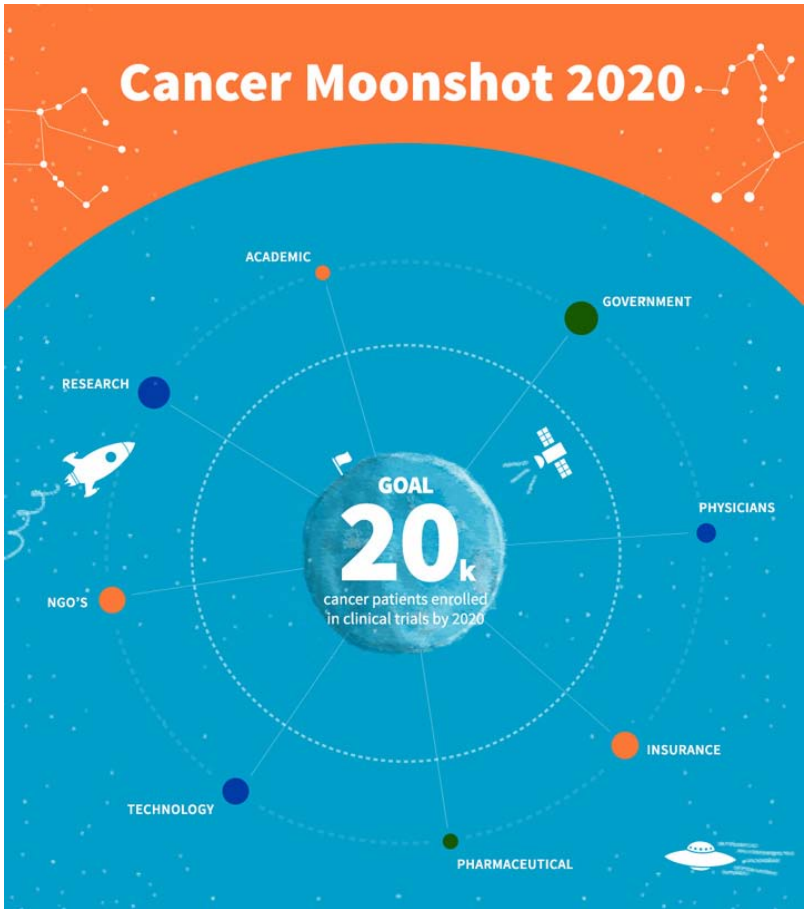
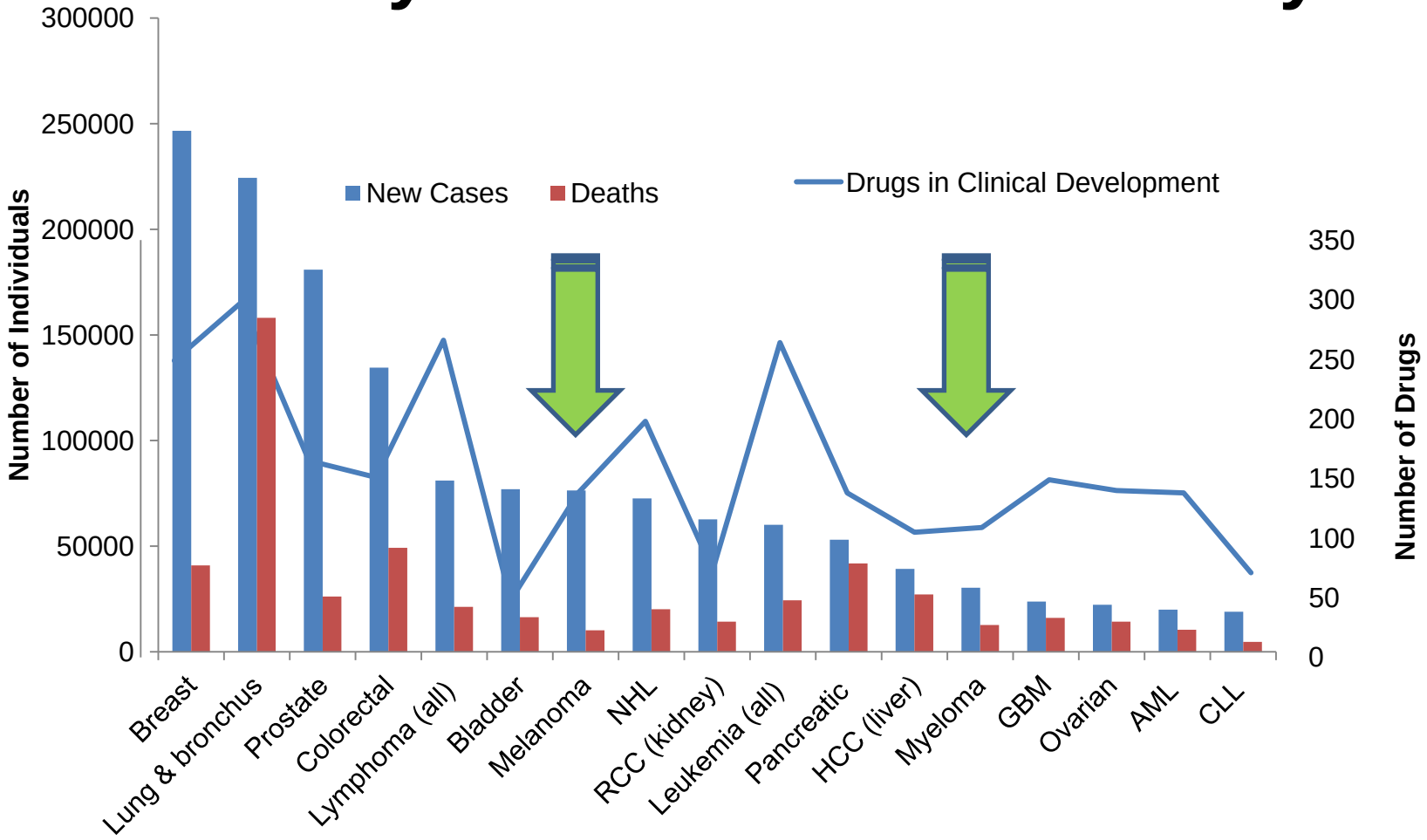
Snaphot (Before the Moonshot) of Where We Are At in Oncology as an Industry



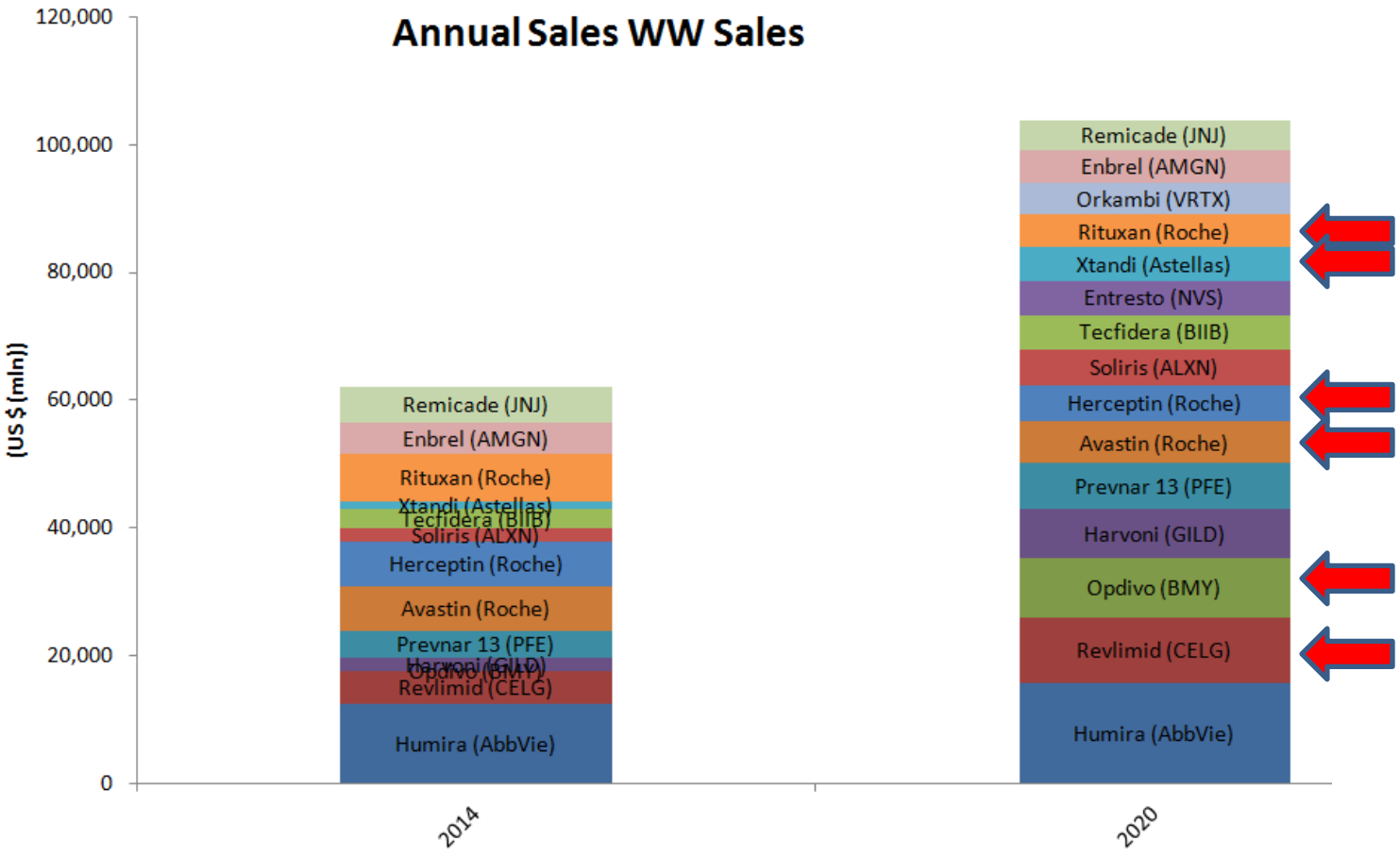
https://www.celgene.com/aiming-moon-cancer-trials/

Cancer Drugs Versus Need: Pipeline Activity vs. Incidence & Mortality



American Cancer Society, Adis R&D Insight

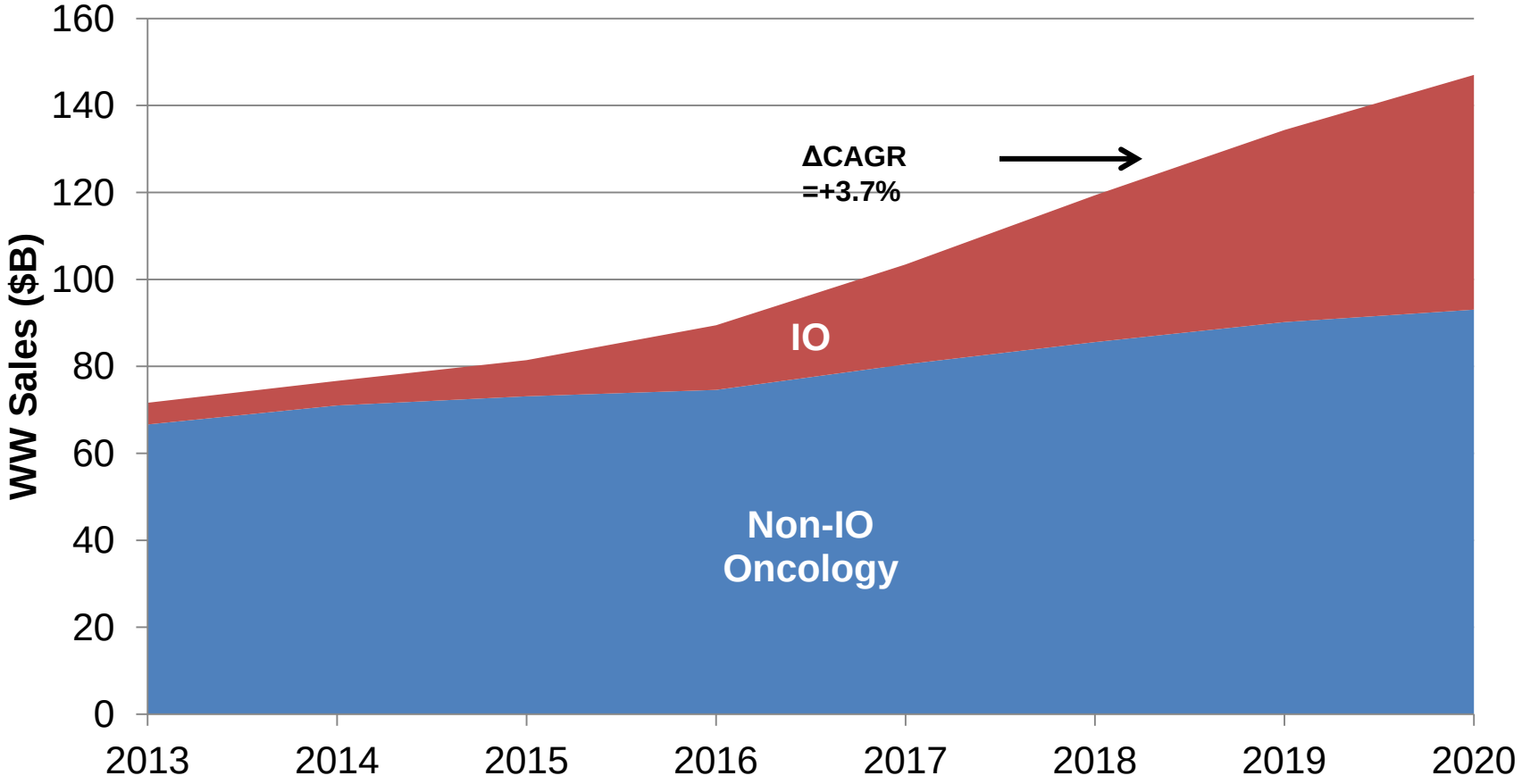
Top 15 Drugs, Sales 2020 (WW)



EvaluatePharma, Defined Health

IO Has Become a Significant Growth Driver in Oncology

Oncology Products WW Sales



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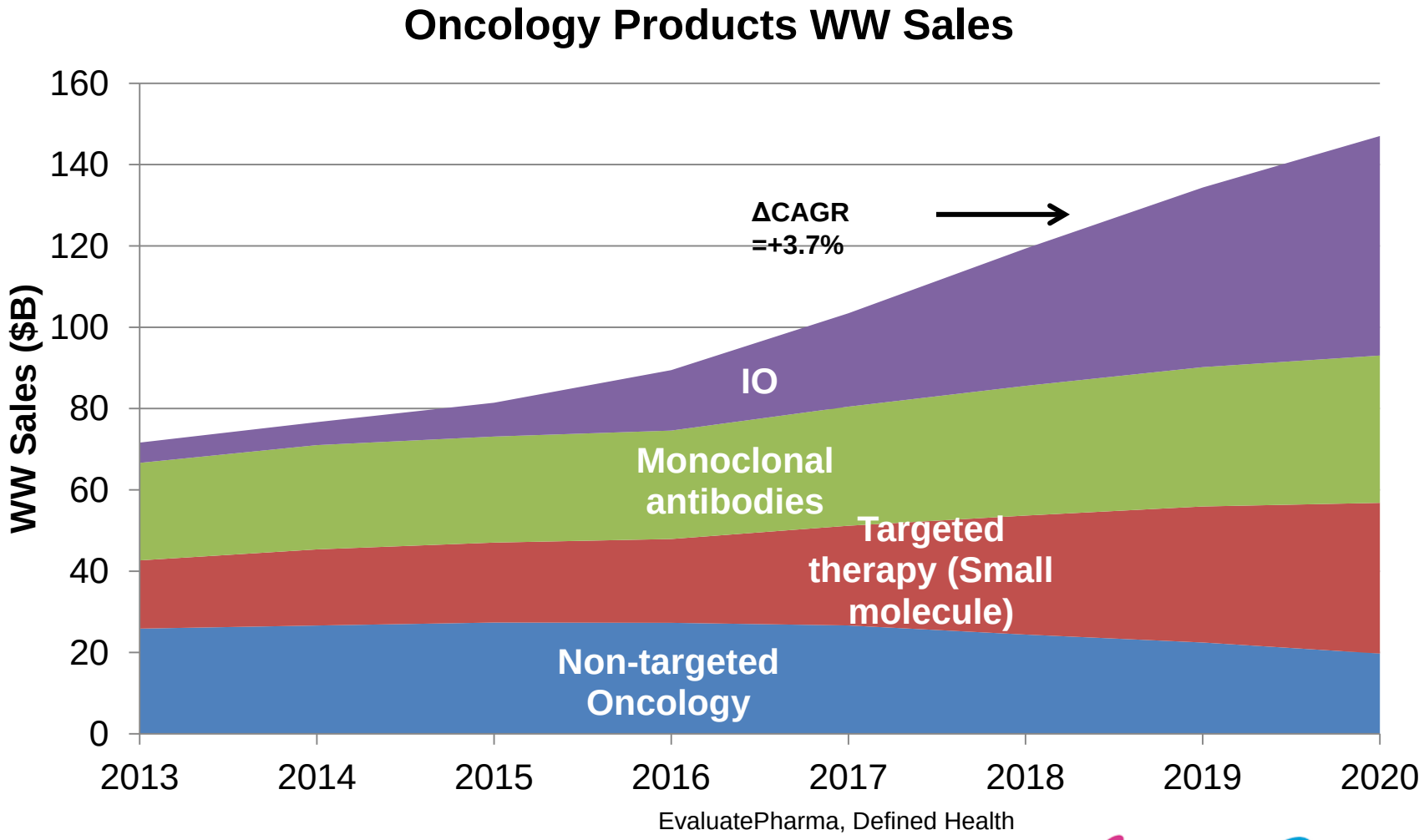
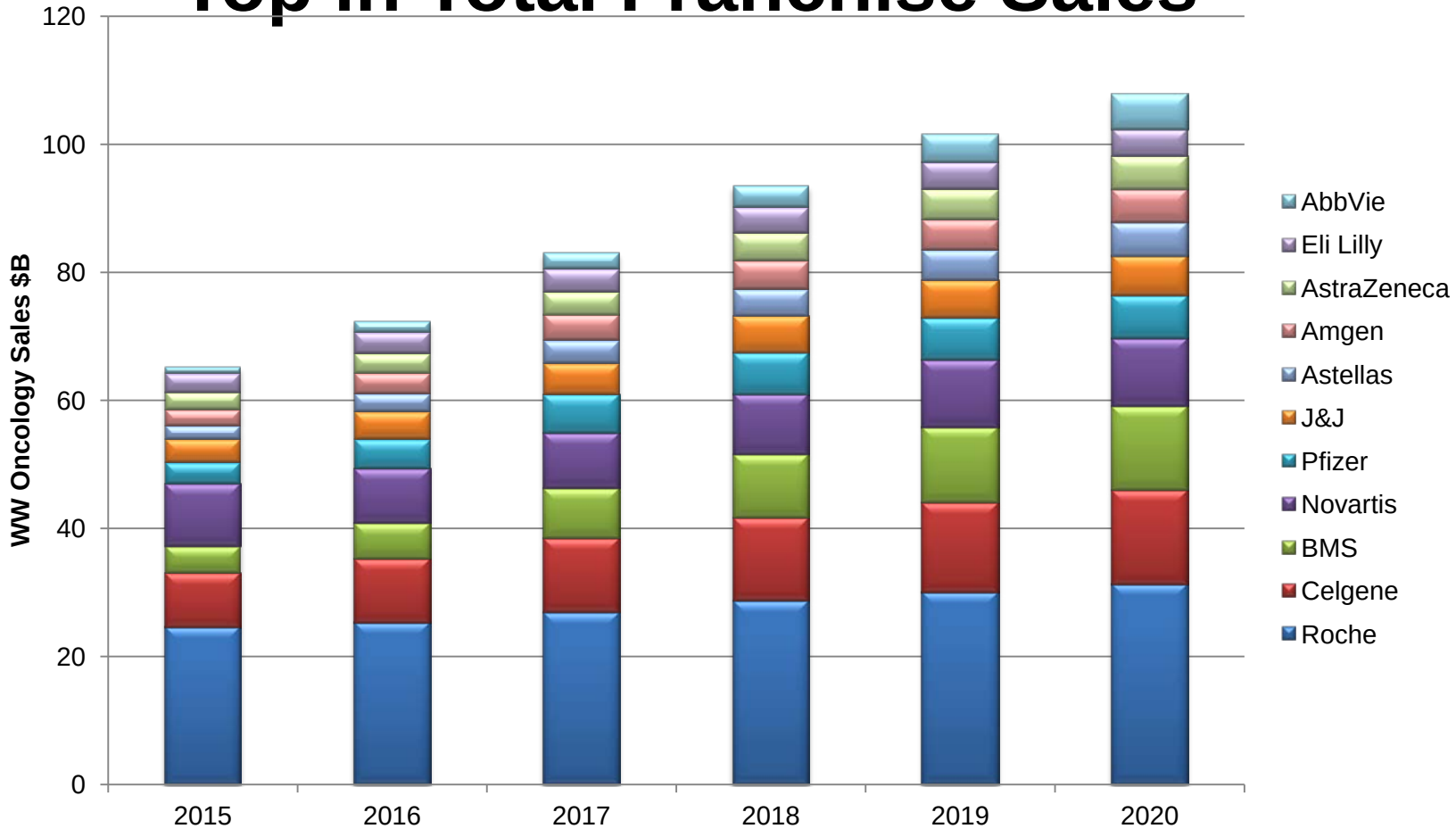
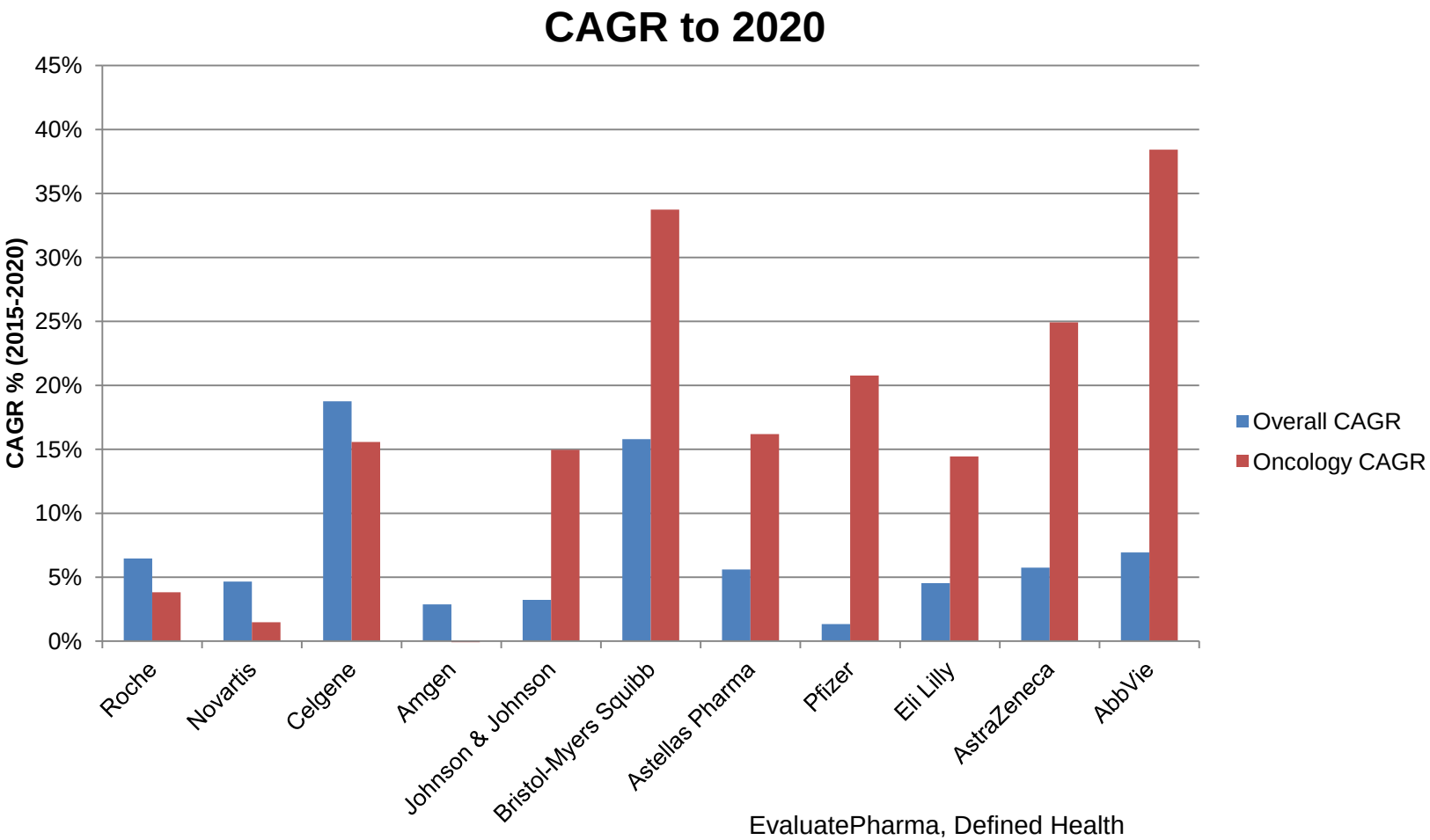


WW Oncology Sales 2015- 2020, By Company – Roche/Genentech Still On Top in Total Franchise Sales



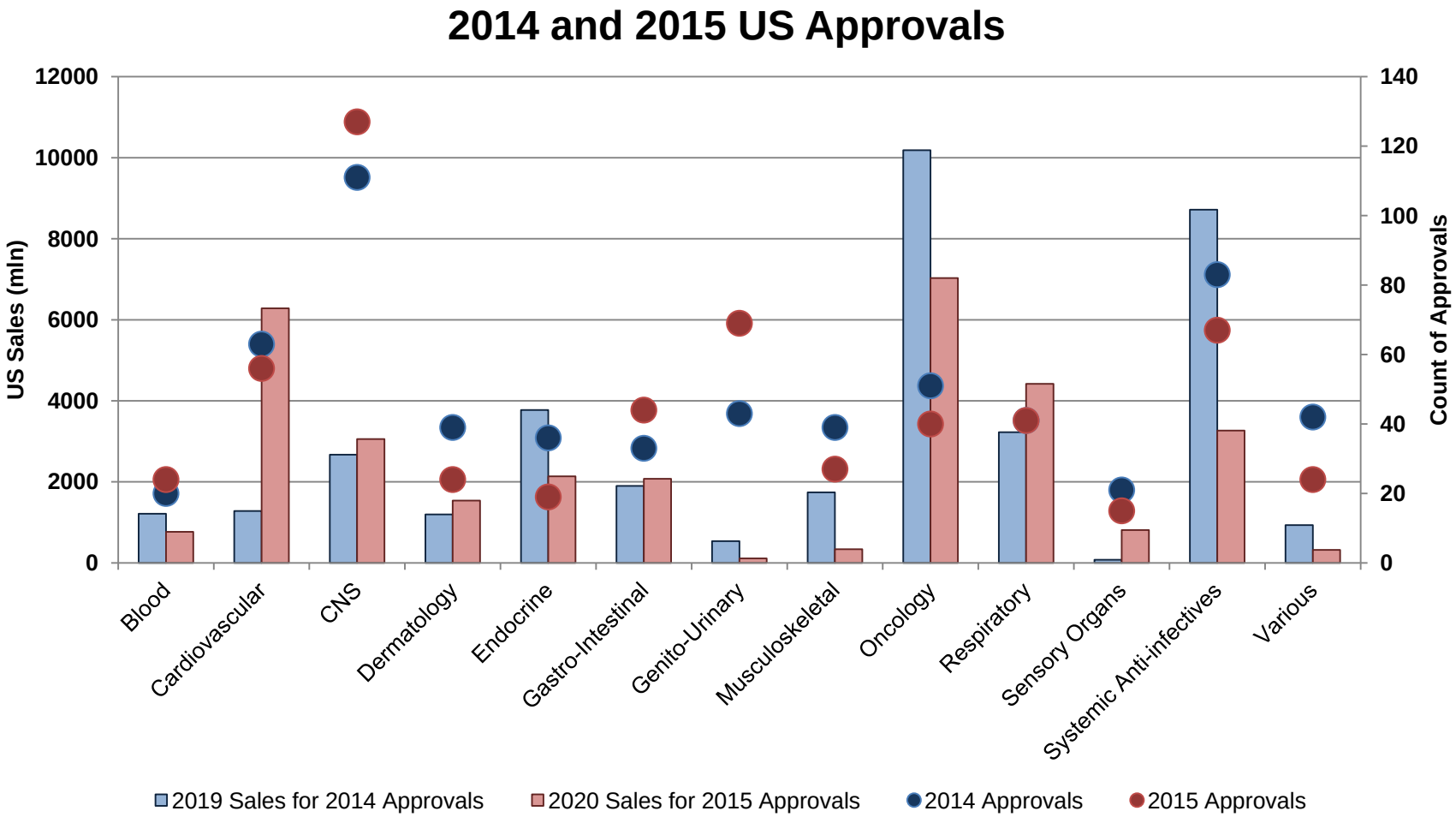
EvaluatePharma, Defined Health

But New IO Launches & Nascent Oncology Franchises Show Most Growth



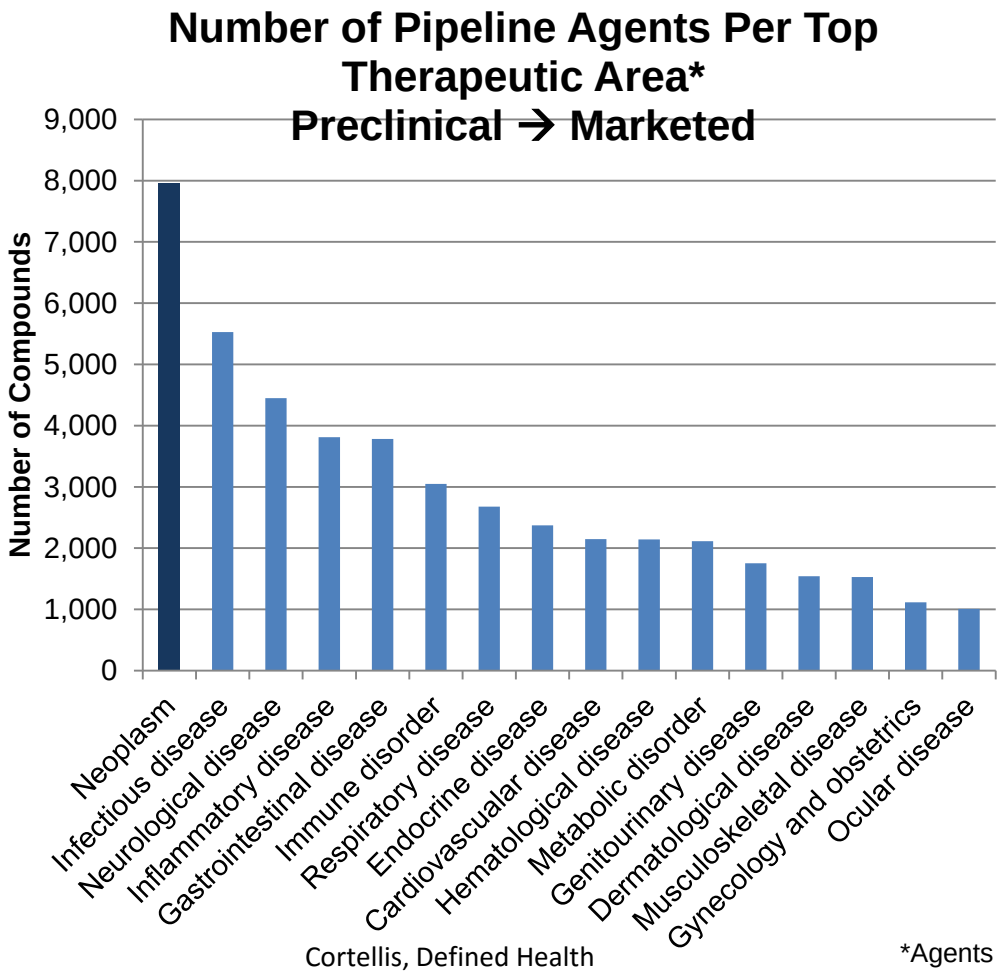
EvaluatePharma, Defined Health

More Punch Per Launch in Oncology

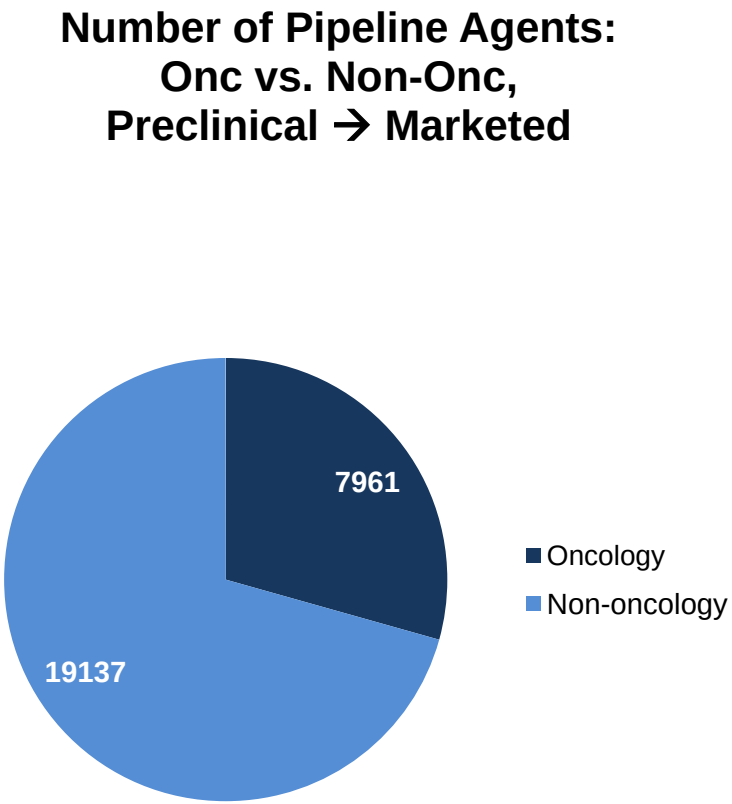


EvaluatePharma, Defined Health

Almost 40% of Pipeline Agents Are for Oncology Indications



Cortellis, Defined Health



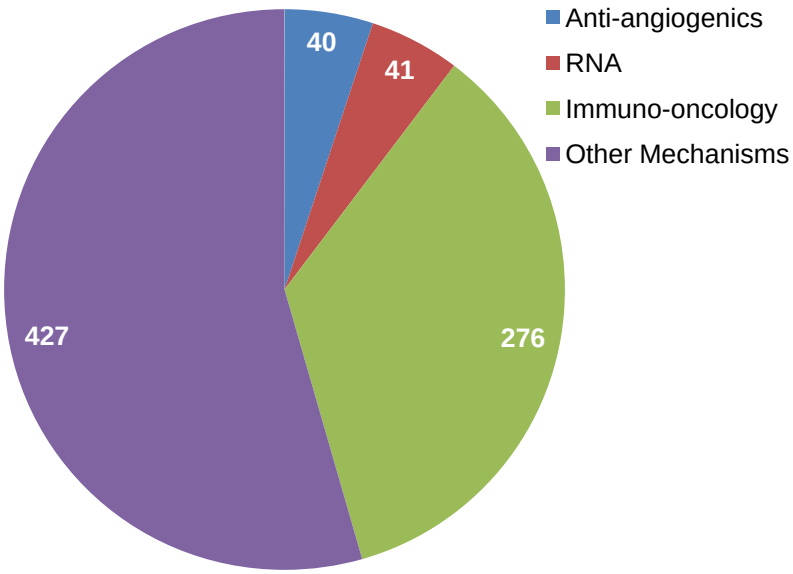
*Agents may be double-counted in multiple categories

Cancer Progress by Defined Health
New York, NY | March 8-9, 2016

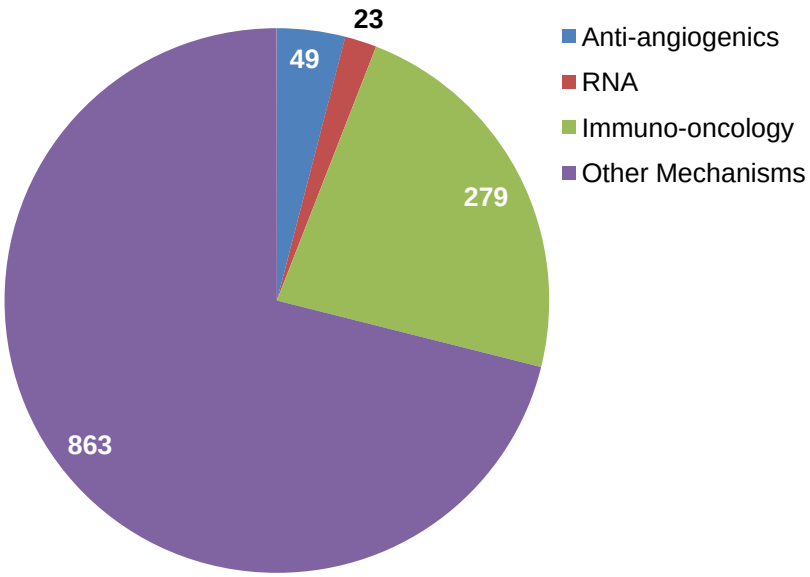
DefinedHealth unconventional insight
THERAPEUTIC INSIGHT by Defined Health
CANCER PROGRESS by Defined Health

Mechanisms in Development

Cancer Pipeline
Phase 2/Phase 3 Development

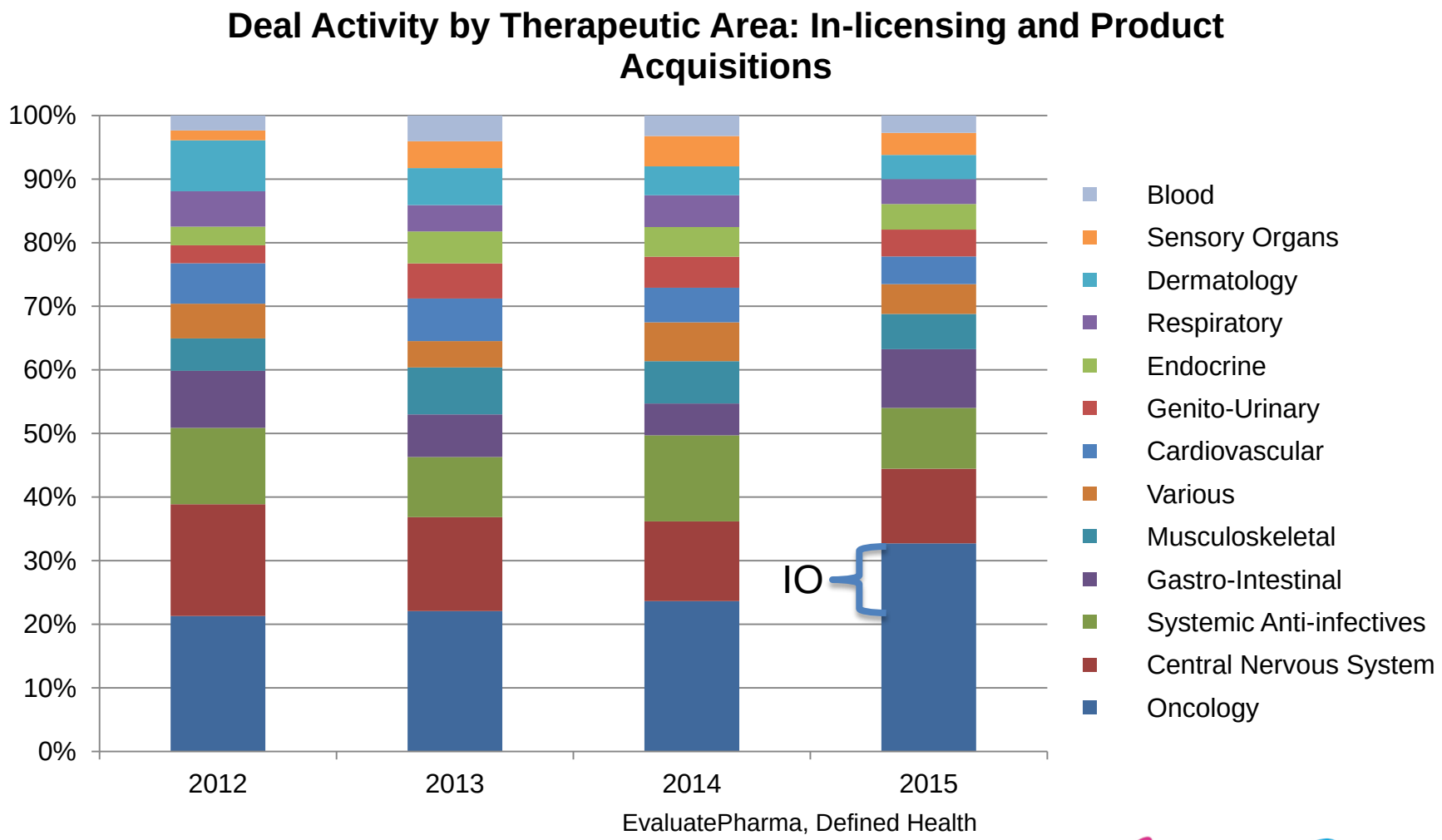


Cancer Pipeline
Phase 1 Development



Cortellis, Defined Health

Oncology Deals Accounted for Over 30% of Deals Across All TAs in 2015



Top Oncology Deals, Jan 2015-Jan 2016

Top 10 Deals by Upfront Value ($M): 2015							
Rank	Company	Deal Partner/ Product Source	Product		Upfront	Milestones	Total
1	Celgene	Juno Therapeutics	JCAR017	IO	1,000	0	1,000
2	Sanofi	Regeneron Pharmaceuticals	REGN2810	IO	640	375	1,015
3	Celgene	AstraZeneca	Durvalumab	IO	450	0	450
4	Medivation	BioMarin Pharmaceutical	Talazoparib		410	160	570
5	Bristol-Myers Squibb	Five Prime Therapeutics	FPA008	IO	350	1,390	1,740
6	Novartis	GlaxoSmithKline	Arzerra S.C.		300	734	1,034
7	AstraZeneca	Innate Pharma	Monalizumab	IO	250	1,025	1,275
8	Novartis	Aduro Biotech	MIW815/STING	IO	250	500	750
9	Celgene	Nurix	Celgene-Nurix Immuno-oncology Program	IO	150	405	555
10	Bristol-Myers Squibb	Bavarian Nordic	Prostvac	IO	140	835	975

Top 10 Deals by Total Value ($M): 2015							
Rank	Company	Deal Partner/ Product Source	Product		Upfront	Milestones	Total
1	Amgen	Xencor	Anti CD3 X CD38 MAb Program	IO	45	1,700	1,745
2	Bristol-Myers Squibb	Five Prime Therapeutics	FPA008	IO	350	1,390	1,740
3	Sanofi	BioNTech	BioNTech-Sanofi mRNA Immunotherapy Project	IO	60	1,500	1,560
4	AstraZeneca	Innate Pharma	Monalizumab	IO	250	1,025	1,275
5	Novartis	GlaxoSmithKline	Arzerra S.C.		300	734	1,034
6	Sanofi	Regeneron Pharmaceuticals	REGN2810	IO	640	375	1,015
7	Eli Lilly	Innovent Biologics	Anti-PD-1 Research Project	IO	0	1,000	1,000
8	Celgene	Juno Therapeutics	JCAR017	IO	1,000	0	1,000
9	Bristol-Myers Squibb	Bavarian nordic	Prostvac	IO	140	835	975
10	Merck KGaA	Intrexon	CAR-T Cell Therapy Research Program	IO	115	826	941

Tumor Heterogeneity & Plasticity

OPINION

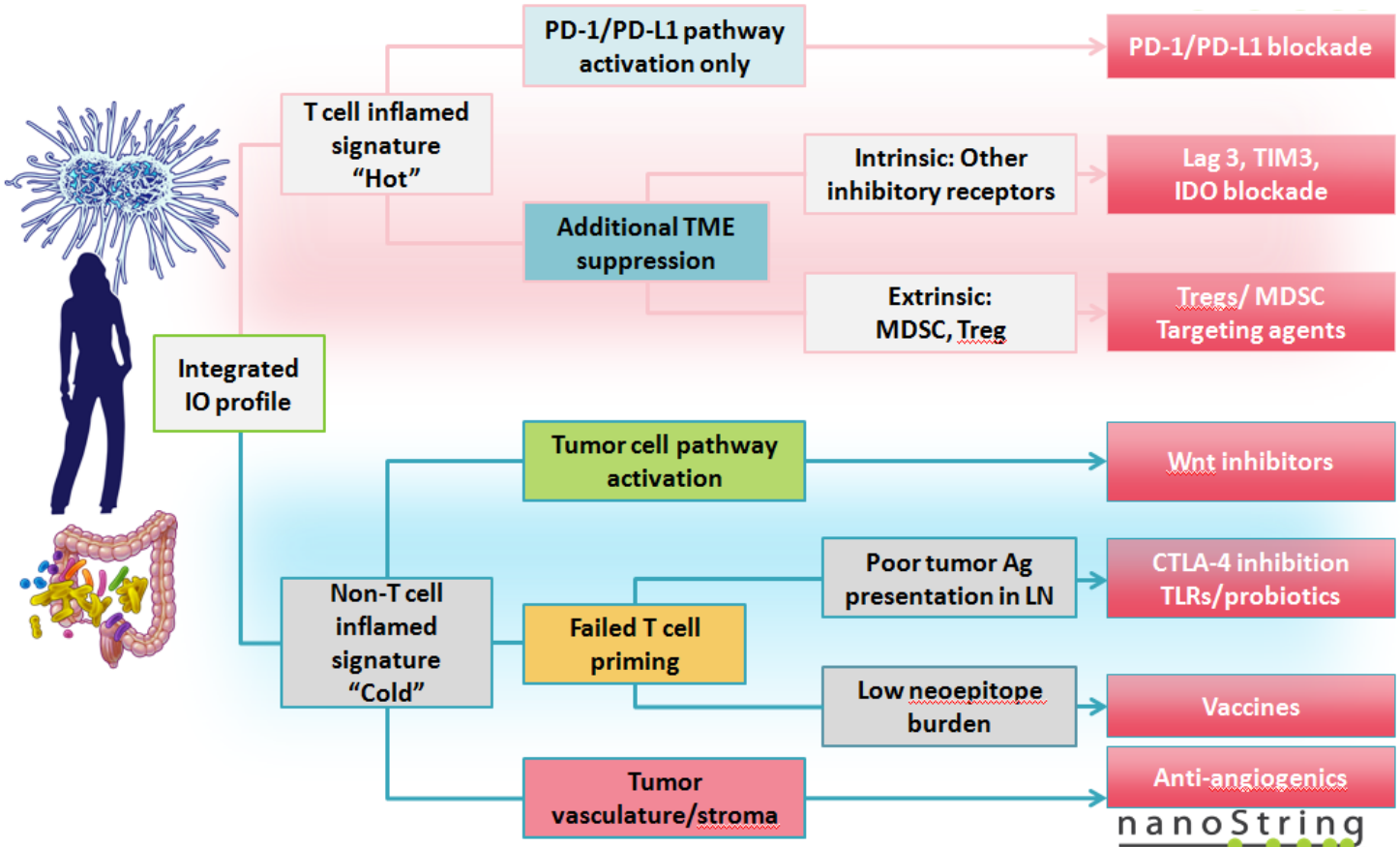
Inducing stable reversion to achieve cancer control

Scott Powers and Robert E. Pollack

Abstract | How can we stop cancer progression? Current strategies depend on modelling progression as the balanced outcome of mutations in, and expression of, tumour suppressor genes and oncogenes. New treatments emerge from successful attempts to tip that balance, but secondary mutational escape from those treatments has become a major impediment because it leads to resistance. In this Opinion article, we argue for a return to an earlier stratagem: tumour cell reversion. Treatments based on selection and analysis of stable revertants could create more durable remissions by reducing the selective pressure that leads to rapid drug resistance.

Zellmer and Zhang, *Cell & Bioscience* 2014, 4:69; Ling et al, *Proc Natl Acad Sci U S A.* 2015 Nov 24;112(47):E6496-505; *Nature Reviews Cancer* March 4, 2016

Is Immunotherapy the Way Forward? (How Better to Counter Tumor Heterogeneity Than the Immune System's Very Own Plasticity!)



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….And Yet

nature REVIEWS CANCER

VIEWPOINT

Acquired resistance to immunotherapy and future challenges

Nicholas P. Restifo, Mark J. Smyth and Alexandra Snyder

Abstract | Advances in immunotherapy have resulted in remarkable clinical responses in some patients. However, one of the biggest challenges in cancer therapeutics is the development of resistant disease and disease progression on or after therapy. Given that many patients have now received various types of immunotherapy, we asked three scientists to give their views on the current evidence for whether acquired resistance to immunotherapy exists in patients and the future challenges posed by immunotherapy.

Parsing the Data, Vetting the Value

Waste in Cancer Drugs Costs $3 Billion a Year, a Study Says

By GARDINER HARRIS MARCH 1, 2016

A nurse prepared a vial of Valcade at Maryland Oncology Hematology office in Rockville, Md., last week. Only 1.3 milligrams of the drug in the 3.5-milligram container was used to treat the patient. The rest was thrown away.
ew York Times

WASHINGTON — The federal Medicare program and private health insurers waste nearly $3 billion every year buying cancer medicines that are thrown out because many drug makers distribute the drugs only in vials that hold too much for most patients, a group of cancer researchers has found.

The expensive drugs are usually injected by nurses working in doctors' offices and hospitals who carefully measure the amount needed for a particular patient and then, because of safety concerns, discard the rest.

thebmj Research Education News & Views Campaigns Archive

Overspending driven by oversized single dose vials of cancer drugs

BMJ 2016 ; 352 doi: http://dx.doi.org/10.1136/bmj.i788 (Published 01 March 2016)
Cite this as: *BMJ* 2016;352:i788

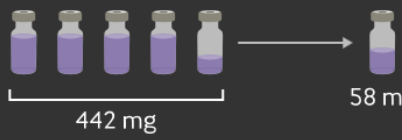
Wasting away

Why cancer drug vial sizes cost billions

Select drug: paclitaxel protein bound (Abraxane)

Year of FDA approval	First approved indication	Dose	Vial sizes available
2005	Breast	260 mg/m²	100 mg

Typical patient dose — Amount wasted — Estimated total cost of leftover drug



$76.7 million

Typical doses calculated based on patient weight of 70kg and skin area of 1.7m².

New York Times, BMJ (British Medical Journal)

Acknowledgments

- James Lee
- Leandra Gerena
- Eva Dixon
- Sam Leyens