APPENDIX A

This table lists the formal definition of different biomarker types as defined by the FDA-NIH Biomarker Working group (2016)

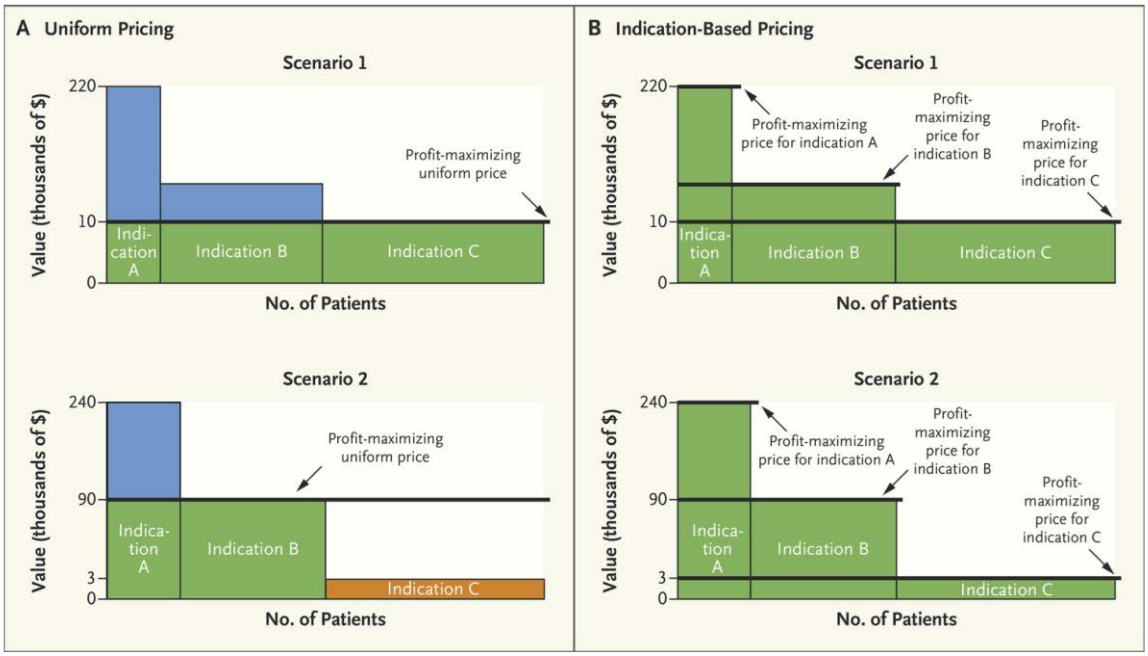
Biomarker type	Official definition	Examples
Diagnostic Biomarker	A biomarker used to detect or confirm presence of a disease or condition of interest or to identify individuals with a subtype of the disease.	1) Sweat chloride may be used as a diagnostic biomarker to confirm cystic fibrosis (Farrell et al. 2008). 2) Glomerular filtration rate (GFR) may be used as a diagnostic biomarker to identify patients with chronic kidney disease (National Kidney Foundation 2002).
Monitoring Biomarker	A biomarker measured serially for assessing status of a disease or medical condition or for evidence of exposure to (or effect of) a medical product or an environmental agent.	1) HIV-RNA may be used as a monitoring biomarker to measure and guide treatment with antiretroviral therapy (ART) (AIDSinfo 2007). 2) Serial measurements of symphysis-fundal height during pregnancy can be used during antenatal screening to detect fetal growth disturbances (Papageorghiou et al. 2016).
Pharmacodynamic / Response Biomarker	A biomarker used to show that a biological response has occurred in an individual who has been exposed to a medical product or an environmental agent.	1) Circulating B lymphocytes may be used as a pharmacodynamic/response biomarker when evaluating patients with systemic lupus erythematosus to assess response to a B-lymphocyte stimulator inhibitor (Stohl and Hilbert 2012). 2) Urinary level of glycosaminoglycans may be used as a pharmacodynamic/response biomarker when evaluating the effect of enzyme replacement therapy for patients with mucopolysaccharidosis type 1 (Jameson et al. 2016).
Predictive Biomarker	A biomarker used to identify individuals who are more likely than similar individuals without the biomarker to experience a favorable or unfavorable effect from exposure to a medical product or an environmental agent.	1) Certain cystic fibrosis transmembrane conductance regulator (CFTR) mutations may be used as predictive biomarkers in clinical trials evaluating treatment for cystic fibrosis, to select patients more likely to respond to particular treatments (Davies et al. 2013). 2) Human leukocyte antigen allele (HLA)–B*5701 genotype may be used as a predictive biomarker to evaluate human immunodeficiency virus (HIV) patients before abacavir treatment, to identify patients at risk for severe skin reactions (AIDSinfo 2007).
Prognostic Biomarker	A biomarker used to identify likelihood of a clinical event, disease recurrence or progression in patients who have the disease or medical condition of interest.	1) Breast Cancer genes 1 and 2 (BRCA1/2) mutations may be used as prognostic biomarkers when evaluating women with breast cancer, to assess the likelihood of a second breast cancer (Basu et al. 2015). 2) Gleason score may be used as a prognostic biomarker when evaluating patients with prostate cancer to assess the likelihood of cancer progression (Epstein et al. 2016; Gordetsky and Epstein 2016).
Safety Biomarker	A biomarker measured before or after an exposure to a medical product or an environmental agent to indicate the likelihood, presence, or extent of toxicity as an adverse effect.	1) Hepatic aminotransferases and bilirubin may be used as safety biomarkers when evaluating potential hepatotoxicity (Senior 2014). 2) Serum creatinine may be used as a safety biomarker

		when evaluating patients on drugs that affect kidney function to monitor for nephrotoxicity (Wasung et al. 2015).
Susceptibility / Risk Biomarker:	A biomarker that indicates the potential for developing a disease or medical condition in an individual who does not currently have clinically apparent disease or the medical condition.	1) Factor V Leiden may be used as a susceptibility/risk biomarker to identify individuals with a predisposition to develop deep vein thrombosis (DVT) (Kujovich 2011). 2) Infection with certain human papillomavirus (HPV) subtypes may be used as a susceptibility/risk bi-omarker to identify individuals with a predisposition to develop cervical cancer (Khan et al. 2005; Schiffman et al. 2011).

Note: Some examples of biomarkers cited in this appendix may be applicable for more than one type of biomarker. For example, in some cases, predictive biomarkers used to identify individuals who are more likely to experience a favorable effect from a drug can also be used as diagnostic biomarkers in the initial detection or confirmation of the disease (e.g. CFTR mutations in Cystic Fibrosis).

APPENDIX B

Effects of uniform pricing versus indication-based pricing.



From Chandra, A. and Garthwaite, C. "The Economics of Indication-Based Drug Pricing." *New England Journal of Medicine*, 377(2), pp.103-106. Copyright © (2017) Massachusetts Medical Society. Reprinted with permission. http://www.nejm.org/doi/full/10.1056/NEJMp1705035

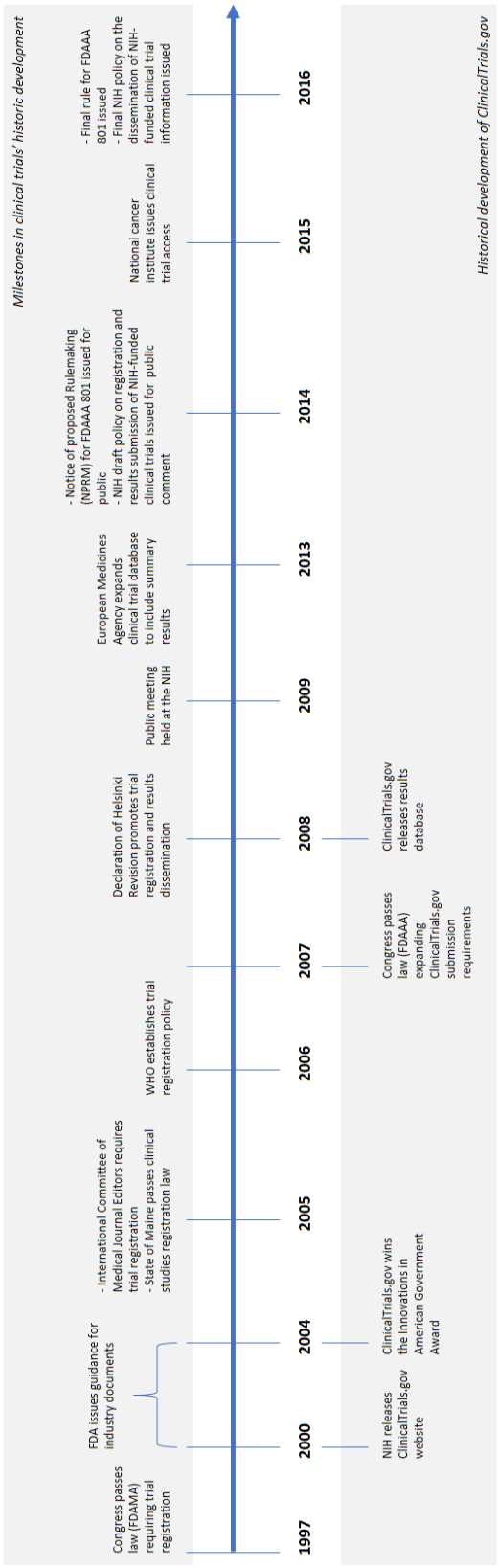
In Panel A, the upper graph represents a uniform-pricing context in which patients with indication A receive the most benefit and those with indication C receive the least. The population with indication C is large, and the value of treatment to this group is close to the value for indication B. As a result, the manufacturer's profit-maximizing price allows all patients to obtain the drug. At this price, the manufacturer earns profits, represented by the green area. However, the firm faces a trade-off. By setting the price in this way, the manufacturer forgoes profits that could be earned by charging higher prices to patients with indications A and B. These forgone profits, represented by the blue areas, are captured by these patients as consumer surplus — the value difference between the most consumers are willing to pay and what they actually pay.

The lower graph in Panel A shows a different scenario, in which the product's valuation for patients with indication C is very low. In this case, it's a better trade-off for the manufacturer to set a high price, at which it knows the payer will allow only patients with indications A and B to obtain the drug. The manufacturer accepts the loss of sales to patients with indication C in exchange for higher profits earned from patients with indications A and B. Comparing these graphs, we see that when the valuation of the product for indication C is relatively low, manufacturers set a higher uniform price, the payer curtails sales to patients with indication C (orange area), and patients with indications A and B obtain less consumer surplus than they did in the first scenario.

Panel B of the graph represents the same set scenarios with respect to the distribution of patients and valuations but allows for indication-based pricing by the manufacturer. The scenario presented is an ex-

treme example in which a monopoly provider is able to set the price exactly at the willingness to pay of the consumer population and thus capture all of the surplus. For scenario 1, the same sets of patients are served but the manufacturer is now able to capture all of the surplus. Scenario 2 represents an output expanding scenario where the manufacturer now finds it profitable to sell to patients with indication C, while also raising the price on the indication A patients that receive the most value from the drug. In total, the introduction of indication-based pricing is shown to weakly increase prices, profits, and the quantity sold.

APPENDIX C


Milestones in clinical trials' historic development
Congress passes law (FDAMA) requiring trial registration
FDA issues guidance for industry documents
- International Committee of Medical Journal Editors requires trial registration
- State of Maine passes clinical studies registration law
WHO establishes trial registration policy
Declaration of Helsinki Revision promotes trial registration and results dissemination
Public meeting held at the NIH
European Medicines Agency expands clinical trial database to include summary results
- Notice of proposed Rulemaking (NPRM) for FDAAA 801 issued for public
- NIH draft policy on registration and results submission of NIH-funded clinical trials issued for public comment
National cancer institute issues clinical trial access
- Final rule for FDAAA 801 issued
- Final NIH policy on the dissemination of NIH-funded clinical trial information issued
1997
2000
2004
2005
2006
2007
2008
2009
2013
2014
2015
2016
NIH releases ClinicalTrials.gov website
ClinicalTrials.gov wins the Innovations in American Government Award
Congress passes law (FDAAA) expanding ClinicalTrials.gov submission requirements
ClinicalTrials.gov releases results database
Historical development of ClinicalTrials.gov

Selected Explanation as provided by the Website of ClinicalTrials.gov (2017):

1997: Congress Passes Law (FDAMA) Requiring Trial Registration
The first U.S. Federal law to require trial registration was the Food and Drug Administration Modernization Act of 1997 (FDAMA) (PDF). Section 113 of FDAMA required the National Institutes of Health (NIH) to create a public information resource on certain clinical trials regulated by the Food and Drug Administration (FDA)

2000: NIH Releases ClinicalTrials.gov Web Site
The first version of ClinicalTrials.gov was made available to the public on February 29, 2000. At the time, ClinicalTrials.gov primarily included NIH-funded studies.

2000–2004: FDA Issues Guidance for Industry Documents
In 2000 FDA issued a draft Guidance for Industry document, which provided recommendations for researchers submitting information to ClinicalTrials.gov. A final guidance document that incorporated comments from the public was issued in 2002.

2004: ClinicalTrials.gov Wins the Innovations in American Government Award
The Innovations in American Government Awards program highlights exemplary models of government innovation and advances efforts to address the Nation's most pressing public concerns.

2005: International Committee of Medical Journal Editors Requires Trial Registration
In 2005 the International Committee of Medical Journal Editors (ICMJE) began requiring trial registration as a condition of publication.

2005: State of Maine Passes Clinical Studies Registration Law (Repealed in 2011)
In 2005 the State of Maine passed a law requiring prescription drug manufacturers or labelers to submit clinical study registration and results information to ClinicalTrials.gov. In 2011 the law was repealed; it is no longer in effect.

2006: World Health Organization Establishes Trial Registration Policy
In 2006 the World Health Organization (WHO) stated that all clinical trials should be registered, and it identified a minimum trial registration dataset of 20 items and in 2007 launched the International Clinical Trials Registry Platform (ICTRP).

2007: Congress Passes Law (FDAAA) Expanding ClinicalTrials.gov Submission Requirements
In 2007 the requirements for submission to ClinicalTrials.gov were expanded after Congress passed the Food and Drug Administration Amendments Act of 2007 (FDAAA). Section 801 of FDAAA (FDAAA 801) required more types of trials to be registered; additional trial registration information; and the submission of summary results, including adverse events, for certain trials. The law also included penalties for noncompliance, such as the withholding of NIH grant funding and civil monetary penalties of up to $10,000 a day.

2008: ClinicalTrials.gov Releases Results Database
In September 2008, as required by FDAAA 801, ClinicalTrials.gov began allowing sponsors and principal investigators to submit the results of clinical studies.[38]

[38] The submission of adverse event information was optional when the results database was released but was required beginning in September 2009.

2008: Declaration of Helsinki Revision Promotes Trial Registration and Results Dissemination
In October, 2008 the 59th World Medical Association (WMA) General Assembly amended the Declaration of Helsinki - Ethical Principles for Medical Research Involving Human Subjects. Two newly added principles (paragraphs 19 and 30) considered the prospective registration and the public disclosure of study results to be ethical obligations.

2009: Public Meeting Held at the National Institutes of Health
In accordance with FDAAA 801, NIH held a public meeting in April 2009 to solicit input from interested individuals about future regulations that will expand the information on ClinicalTrials.gov.

2013: European Medicines Agency Expands Clinical Trial Database to Include Summary Results
In October 2013 the European Medicines Agency (EMA) released a new version of the European Clinical Trials Database (EudraCT). Notably, the EudraCT summary results data requirements are "substantially aligned" with those of the ClinicalTrials.gov results database.

2014: Notice of Proposed Rulemaking (NPRM) for FDAAA 801 Issued for Public Comment
In November 2014 the U.S. Department of Health and Human Services issued a notice of proposed rulemaking (NPRM) describing the proposed requirements and procedures for registering and submitting the results, including adverse events, of clinical trials on ClinicalTrials.gov, in accordance with FDAAA 801.

2014: NIH Draft Policy on Registration and Results Submission of NIH-Funded Clinical Trials Issued for Public Comment.
In November 2014 NIH proposed a policy to ensure that every clinical trial (see the Revised NIH Definition of "Clinical Trial") that receives NIH funding is registered on ClinicalTrials.gov and has summary results submitted and posted in a timely manner, whether subject to FDAAA 801 or not.

2015: National Cancer Institute Issues Clinical Trial Access Policy
In January, 2015 the NIH National Cancer Institute (NCI) issued its Policy Ensuring Public Availability of Results from NCI-supported Clinical Trials. The policy states, "Final Trial Results are expected to be reported in a publicly accessible manner within twelve (12) months of the Trial's Primary Completion Date regardless of whether the clinical trial was completed as planned or terminated earlier."

2016: Final Rule for FDAAA 801 Issued
In September 2016, the U.S. Department of Health and Human Services issued a Final Rule for Clinical Trials Registration and Results Information Submission (42 CFR Part 11) that clarifies and expands the regulatory requirements and procedures for submitting registration and summary results information of clinical trials on ClinicalTrials.gov, in accordance with FDAAA 801. The final rule is intended to make it clear to sponsors, investigators, and the public which trials must be submitted, when they must be submitted, and whether compliance has been achieved.

2016: Final NIH Policy on the Dissemination of NIH-funded Clinical Trial Information Issued
In September 2016, NIH issued a final policy to promote broad and responsible dissemination of information from NIH-funded clinical trials through ClinicalTrials.gov. Under this policy, every clinical trial funded in whole or in part by NIH is expected to be registered on ClinicalTrials.gov and have summary results information submitted and posted in a timely manner, whether subject to FDAAA 801 or not.

APPENDIX D

ICD-9 Sub-chapter	Number of trials	Neoplasm (cancer) Sub-chapter
Intestinal Infectious Diseases	402	No
Tuberculosis	414	No
Zoonotic Bacterial Diseases	80	No
Other Bacterial Diseases	1749	No
Human Immunodeficiency Virus (HIV) Infection	2909	No
Poliomyelitis And Other Non-Arthropod-Borne Viral Diseases And Prion Diseases Of Central Nervous System	232	No
Viral Diseases Generally Accompanied By Exanthem	627	No
Arthropod-Borne Viral Diseases	210	No
Other Diseases Due To Viruses And Chlamydiae	3344	No
Rickettsioses And Other Arthropod-Borne Diseases	174	No
Syphilis And Other Venereal Diseases	74	No
Other Spirochetal Diseases	14	No
Mycoses	663	No
Helminthiases	86	No
Other Infectious And Parasitic Diseases	532	No
Late Effects Of Infectious And Parasitic Diseases	3	No
Malignant Neoplasm Of Lip, Oral Cavity, And Pharynx	468	Yes
Malignant Neoplasm Of Digestive Organs And Peritoneum	8793	Yes
Malignant Neoplasm Of Respiratory And Intrathoracic Organs	5891	Yes
Malignant Neoplasm Of Bone, Connective Tissue, Skin, And Breast	9034	Yes
Malignant Neoplasm Of Genitourinary Organs	7110	Yes
Malignant Neoplasm Of Other And Unspecified Sites	9340	Yes
Malignant Neoplasm Of Lymphatic And Hematopoietic Tissue	8981	Yes
Neuroendocrine Tumors	382	Yes
Benign Neoplasms	440	Yes
Carcinoma In Situ	1	Yes
Neoplasms Of Uncertain Behavior	2377	Yes
Neoplasms Of Unspecified Nature	2312	Yes
Disorders Of Thyroid Gland	135	No
Diseases Of Other Endocrine Glands	6639	No
Nutritional Deficiencies	526	No
Other Metabolic And Immunity Disorders	5532	No
Diseases Of The Blood And Blood-Forming Organs	3392	No
Psychoses	2855	No
Neurotic Disorders, Personality Disorders, And Other Nonpsychotic Mental Disorders	4348	No
Intellectual Disabilities	5	No
Inflammatory Diseases Of The Central Nervous System	150	No
Organic Sleep Disorders	257	No

Hereditary And Degenerative Diseases Of The Central Nervous System	3541	No
Pain	228	No
Other Headache Syndromes	33	No
Other Disorders Of The Central Nervous System	2466	No
Disorders Of The Peripheral Nervous System	1024	No
Disorders Of The Eye And Adnexa	2440	No
Diseases Of The Ear And Mastoid Process	393	No
Acute Rheumatic Fever	1	No
Chronic Rheumatic Heart Disease	110	No
Hypertensive Disease	1378	No
Ischemic Heart Disease	1933	No
Diseases Of Pulmonary Circulation	613	No
Other Forms Of Heart Disease	2515	No
Cerebrovascular Disease	1285	No
Diseases Of Arteries, Arterioles, And Capillaries	1179	No
Diseases Of Veins And Lymphatics, And Other Diseases Of Circulatory System	1605	No
Acute Respiratory Infections	455	No
Other Diseases Of The Upper Respiratory Tract	1047	No
Pneumonia And Influenza	1794	No
Chronic Obstructive Pulmonary Disease And Allied Conditions	3159	No
Pneumoconioses And Other Lung Diseases Due To External Agents	18	No
Other Diseases Of Respiratory System	914	No
Diseases Of Oral Cavity, Salivary Glands, And Jaws	841	No
Diseases Of Esophagus, Stomach, And Duodenum	1040	No
Appendicitis	20	No
Hernia Of Abdominal Cavity	20	No
Noninfectious Enteritis And Colitis	1213	No
Other Diseases Of Intestines And Peritoneum	993	No
Other Diseases Of Digestive System	1576	No
Nephritis, Nephrotic Syndrome, And Nephrosis	1508	No
Other Diseases Of Urinary System	1207	No
Diseases Of Male Genital Organs	793	No
Disorders Of Breast	37	No
Inflammatory Disease Of Female Pelvic Organs	816	No
Other Disorders Of Female Genital Tract	1454	No
Ectopic And Molar Pregnancy	12	No
Other Pregnancy With Abortive Outcome	91	No
Complications Mainly Related To Pregnancy	396	No
Normal Delivery, And Other Indications For Care In Pregnancy, Labor, And Delivery	130	No
Complications Occurring Mainly In The Course Of Labor And Delivery	20	No
Complications Of The Puerperium	84	No
Infections Of Skin And Subcutaneous Tissue	205	No

Other Inflammatory Conditions Of Skin And Subcutaneous Tissue	2100	No
Other Diseases Of Skin And Subcutaneous Tissue	1536	No
Arthropathies And Related Disorders	3237	No
Dorsopathies	545	No
Rheumatism, Excluding The Back	1220	No
Osteopathies, Chondropathies, And Acquired Musculoskeletal Deformities	982	No
Congenital Anomalies	789	No
Maternal Causes Of Perinatal Morbidity And Mortality	4	No
Other Conditions Originating In The Perinatal Period	155	No
Symptoms	6901	No
Nonspecific Abnormal Findings	402	No
Ill-Defined And Unknown Causes Of Morbidity And Mortality	195	No
Fractures	134	No
Sprains And Strains Of Joints And Adjacent Muscles	22	No
Intracranial Injury, Excluding Those With Skull Fracture	226	No
Internal Injury Of Thorax, Abdomen, And Pelvis	83	No
Open Wounds	252	No
Injury To Blood Vessels	7	No
Late Effects Of Injuries, Poisonings, Toxic Effects, And Other External Causes	3	No
Superficial Injury	28	No
Contusion With Intact Skin Surface	15	No
Burns	119	No
Injury To Nerves And Spinal Cord	204	No
Certain Traumatic Complications And Unspecified Injuries	138	No
Poisoning By Drugs, Medicinal And Biological Substances	60	No
Toxic Effects Of Substances Chiefly Nonmedicinal As To Source	78	No
Other And Unspecified Effects Of External Causes	2264	No
Complications Of Surgical And Medical Care, Not Elsewhere Classified	515	No
Persons With Potential Healthhazards Related To Communicable Diseases	54	No
Persons With Need For Isolation, Other Potential Health Hazards And Prophylactic Measures	41	No
Persons With Potential Health Hazards Related To Personal And Family History	16	No
Persons Encountering Health Services In Circumstances Related To Reproduction And Development	233	No
Persons With A Condition Influencing Their Health Status	835	No
Persons Encountering Health Services For Specific Procedures And Aftercare	31	No
Persons Without Reported Diagnosis Encountered During Examination And Investigation Of Individuals And Populations	214	No

APPENDIX E

Identifying publicly listed firms

In order to understand the "lineage" (ownership histories) of firms, we take advantage of data on a firm's "Ancestor" as provided by the Thompson Reuters Permanent Identifier ("PermID") database. Thompson Reuters describes the database as "a machine-readable identifier developed to create a unique reference for any data item" noting that a "PermID provides comprehensive identification across a wide variety of entity types including organizations, instruments, funds, issuers and people."[39] We match firms in the Cortellis data to the firms' PermIDs: 90.0% of the companies in the Cortellis database have PermID information (137,160 out of 152,357). Of the137,160 companies with PermIDs we matched 99.2% of them with the PermID data. This results in firm-specific data on whether or not a firm is publicly listed. The same database also allows us to observe if a firm has been acquired by a publicly listed firm ("ancestor"). Based on a combination of trial date (from Cortellis) and acquisition data (from the PermID database), we can understand whether a trial was sponsored by a publicly listed firm (*and/) or* whether or not the sponsor was a subsidiary of a publicly listed firm.

As a result of the data considerations described below, we assign upper and lower-bound measures of whether or not a firm was publicly listed at the time of an observed clinical trial as follows.

Firms

	Firm	Ancestor	$Public_0$	$Public_1$	$Public_2$	$Public_3$
		Ancestor (AKA parent) firm observed at time = T	Firm or its ancestor is publicly traded on trial date (unobserved *true* status)	Firm is publicly traded (observed at time = T)	Ancestor is publicly traded (observed at time = T)	Either $Public_1$ or $Public_2$ is TRUE
1.	Pfizer Inc	Pfizer Inc	TRUE	TRUE	TRUE	TRUE
2.	Pfizer Inc (India)	Pfizer Inc	TRUE	FALSE	TRUE	TRUE
3.	Small Bio Corp.	GSK	FALSE	FALSE	TRUE	TRUE

[39] More detail can be found at https://financial.thomsonreuters.com/en/products/data-analytics/market-data/reference-data/permid-data-management.html

4.	Genentech	Roche	TRUE	FALSE	TRUE	TRUE
5.	Xenoport	Arbor Pharmaceuticals	TRUE	FALSE	FALSE	FALSE
6.	ALK-Abello	Lundbeck Foundation	TRUE	TRUE	FALSE	TRUE

We use ancestor firms' public status instead of firms' (own) public status assigning legitimate subsidiaries to their parent company's status as wanted (Row 2); however, this method also assigns some acquired firms to an incorrect status.

In Row 3 above, Small Bio Corp. conducts a trial as a privately owned firm at time 0 and is acquired by GSK at time t > 0. Due to data limitations we observe only the most recent firm ancestor (GSK) at time of data collection T > t > 0, and thus the ancestor's public status at time T (TRUE) misrepresents Small Bio's status on the trial date. This is not an issue for firms that were publicly traded before being acquired as long as the acquiring firm is public as well (as in the example in Row 4). This is, however, a complication for firms that were publicly traded and then "delisted" after being purchased by a private firm (as in the example in Row 5).

Rarely, firms are listed as public with non-publicly traded ancestors. This generally indicates partial private ownership of a public firm (as in the example in Row 6).

None of the measures of $Public_j|_{j\in 1,2,3}$ match the unobserved true public status ($Public_0$) for each case, but they can still be useful in a bounding exercise. Because $Public_1$is never TRUE in any case that $Public_0$ is FALSE, it can be used as a lower bound for $Public_0$.

Measure 3 is NOT an upper bound on Measure 0 because, as is the case with Xenoport, $Public_0 = TRUE$ does not imply $Public_3 = TRUE$. However, the true share of trials run by public firms will be bounded above by Measure 3 share as long as there are more trials misclassified as public (due to a later acquisition) than misclassified as private. This is proven below:

$$Share\ Public_3$$

$$= \frac{\#Public\ Trials + \#Misclassified_{Private \to Public} - \#Misclassified_{Public \to Private}}{\#Trials}$$

$$If\ \#Misclassified_{Private \to Public} > \#Misclassified_{Public \to Private} \Rightarrow$$

$$Share\ Public_3 > \frac{\#Public\ Trials}{\#Trials} = Share\ Public_0$$

So in this case, $Share\ Public_3$ is an upper bound on the true share of trials funded by public firms.

We cannot directly measure the number of misclassified trials to test whether this assumption holds, but because these misclassifications result from mergers and acquisitions, public firms acquiring private firms will likely make up the lion's share of such activity and the bound will hold.

The process by which we calculate dummy variables indicating whether a trial is public by the different measures is outlined below:

1. For each firm
    a. $Public_1 = \mathbf{1}(firm\ is\ public\ in\ 2017)$;
    b. $Public_2 = \mathbf{1}(firm's\ ancestor\ is\ public\ in\ 2017)$
2. For each trial and firm recode
    a. $Public_1 = 0\ if\ IPO\ Date > Trial\ Date$.
    b. $Public_2 = 0\ if\ Ancestor\ IPO\ Date > Trial\ Date$.
3. For each firm-ancestor pair calculate $Public_3 = \max\{Public_1, Public_2\}$.
4. For each trial, calculate whether any public firms were involved with the trial:
    a. $Public\ Trial_1 = \max\left\{\{Public_{1j}: j \in J\}\right\}$
    b. $Public\ Trial_3 = \max\left\{\{Public_{3j}: j \in J\}\right\}$

       $for\ the\ set\ J\ of\ firm - ancestor\ pairs\ involved\ with\ the\ trial$

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