National Kidney Cancer Association

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February 28, 1992

Senator Edward M. Kennedy
Senate Committee on Labor and Human Resources
428 Dirksen Senate Office Building
Washington, DC 20510

Dear Senator Kennedy:

It is my deep concern that the Committee on Labor and Human Resources will change the Orphan Drug Act in ways that will hurt patients. Since I cannot come to Washington and testify before the Committee, I am writing this letter. Please read it to the Committee and give copies to all members of the Committee.

By way of introduction, kidney cancer is a rare disease accounting for slightly over 2 percent of all cancer cases. This year, there will be about 25,000 new cases of kidney cancer in the United States. Also 10,600 Americans will die from kidney cancer -- about one death every 48 minutes.

Even though kidney cancer is a rare "orphan" disease, the American death rate from kidney cancer is twice the death rate of the Vietnam War. However, unlike the War, kidney cancer goes right on killing.

As President of the National Kidney Cancer Association, I know many people who have this disease, and I am a kidney cancer patient myself. I have started several high tech companies as well as being a founder of the Association. I hold a Ph.D. in marketing and finance from Northwestern University and I have been on the faculty at Northwestern and the University of Illinois.

One of my companies tracks R&D expenditures and publishes statistical references works on corporate financial performance. These works are sold to research centers such as Bell Labs, Wall Street, and major institutions including the Federal government.

I do not believe that S.2060 would be beneficial. The Orphan Drug Act is working and Congress deserves much credit for this Act. It would be a mistake to reduce the economic incentives which Congress created to stimulate research and marketing of drugs for orphan diseases.

I know that many people who have orphan diseases dislike the high prices of the drugs which have been developed under the Orphan Drug Act. However, these people do not understand the laws of economics. Nor do they understand R&D and marketing. They view the price only from a consumer point of view, not from a public policy perspective.

The only people who complain about drug prices are people who have a drug for their particular disease. However, these people have short memories. They fail to remember, that at one time, there was no drug for their disease. In many instances, they would have no drug without the Orphan Drug Act.

If Congress removes incentives for orphan drug development, such as temporary monopolies, the people who have already benefitted from the Act and have a drug as a result of the Act will benefit still more. Competition will increase, and prices will fall in markets for existing orphan drugs.

At the same time, people who have orphan diseases but no drug will be put at a disadvantage. With reduced incentives, companies will be less aggressive in developing drugs for these diseases. Many Americans with serious illness will never have a drug for their condition.

As a piece of legislation, the Orphan Drug Act was designed to increase the number of drugs available for different diseases. Much evidence indicates that it is doing this job.

While the Act grants companies temporary market monopolies for orphan diseases, it does not reduce the high cost of drug development. Often, it is more expensive to develop an orphan drug than a regular drug. For example, when drugs are developed, it is often necessary to obtain research information or tissue or blood samples from people who have the disease. Since people with an orphan disease are rare, it can be very difficult and costly to obtain these inputs to the research process. At the very least, an orphan drug requires the same extensive research as a regular drug.

Moreover, an orphan drug must pass through the same FDA regulatory process as a regular drug. Complying with FDA regulations and going through the approval process is often more expensive for an orphan drug.

One reason for higher costs is that the FDA requires that clinical trials be performed to show proof of efficacy and safety. However, because an orphan disease is rare, conducting such clinical trials can be very difficult and costly.

No one medical institution may have enough patients to do a clinical trial. Multiple institutions need to be recruited and managed in order to get enough data to satisfy FDA requirements. In addition, many orphan diseases are chronic diseases and many are related to genetic factors. Such diseases may require extra long clinical trials to measure efficacy and observe side effects.

For example, about 70 percent of kidney cancer patients have no visible spread of the disease beyond the kidney at time of diagnosis. A surgeon removes the diseased kidney. No further treatment is done because chemotherapy and radiation offer no additional benefit in these cases. However, 50 percent of all such surgically treated patients have recurrence of their cancer. Half will have recurrence within 5 years and half will have recurrence after 5 years. As you can readily see, any orphan drug aimed at preventing recurrence would have to be tested for years and years before efficacy could be measured.

A good case history for the Committee to study is Interleukin-2 which became the first FDA approved treatment for kidney cancer on January 17, 1992.

Interleukin-2 was first identified as an anti-cancer agent in 1976. It was first tested in humans in 1984. The first FDA hearing to review Cetus's application for marketing the drug was in July 1990. The FDA turned the application down because there wasn't enough research data to warrant approval.

After eighteen more months of research--with over 850 kidney cancer patients dying per month--it was approved by the FDA in January 1992. During this eighteen month period, Cetus had to fire 10 percent of its employees and had to merge with Chiron in order to survive.

Roche Laboratories, which had its own version of IL-2 under development, decided to withdraw from the market after the FDA failed to approve Cetus's application for marketing at the July 30, 1990 hearing. See the enclosed letter from Roche.

Ortho Pharmaceuticals had also obtained a license to market IL-2, but decided not to market the product.

The development cost of Interleukin-2 is reported at over $ 120 million. According to the company, it had to file over 85,000 pages of documents with the FDA to get the drug through the approval process.

The reason that I know so much about IL-2 is that I testified before the Biological Response Modifiers Advisory Committee to the FDA in July of 1990 and again in January 1992. I also conducted a research study of IL-2 as a treatment for kidney cancer and surveyed the Society of Urologic Oncology, the nation's 100 leading urologic cancer experts. Over 80 percent had actually tested the drug with patients, and 70 percent thought it was the best available treatment.

Yet, today, only one company makes the drug, Cetus, now part of Chiron. And we came close to having no company supplying the drug. In all its years as a venture capital startup and as an independent public company, Cetus never made any money. Its total annual sales during recent years were only about $ 30 million. If Cetus had gone under, there would be no IL-2.

Roche Pharmaceuticals
a division of Hoffmann-La Roche Inc.

340 Kingsland Street
Nutley, New Jersey 07110-1199

Direct Dial 201-235-2933

August 5, 1991

Nicholas J. Vogelzang, M.D.
Assistant Professor
U. of Chicago Medical Center
Box 420
5841 S Maryland Avenue
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Dear Doctor Vogelzang:

I am writing to inform you of the decision by Hoffmann-La Roche Inc. to discontinue further development of IL-2 (Teceleukin). All investigational activities will be phased out, and supplies of drugs for further clinical testing will no longer be available. It should be emphasized that this decision is not due in any way to safety or tolerability issues.

We realize that some studies are still ongoing and that there are patients currently being treated. Manufacture of IL-2 has ceased. However, there is existent stock which, depending on actual demand, may be sufficient to allow for the continued treatment of such patients for a period of several months.

Therefore, please inform me of the status of any ongoing studies so that existing supplies of IL-2 can be appropriately allocated.

We are working towards phasing out our IL-2 activities as smoothly as possible. Your support of IL-2 studies is greatly appreciated and we regret any difficulties this decision may incur.

Very truly yours,

Edward Schnipper

Edward Schnipper, M.D.
Director
Hematology/Oncology

ES/ca

I mention these things because many people believe that the drug industry is wildly profitable. Many people forget the failures--the companies and products which don't survive. There are very significant risks in drug research. On a risk adjusted basis, I don't believe the drug industry is so profitable.

Since I testified before the FDA regarding IL-2, I have enclosed copies of my testimony.

I absolutely believe that the regulatory policies of the Federal government significantly raised the cost of Interleukin-2 for patients. It should be noted that as far I could determine, the Orphan Drug Office within the FDA was sympathetic to patients, but totally ineffective in helping IL-2 move through the agency.

Even when a drug is an orphan drug and protected from competition for a period of time, the company must still recover its development costs. With a small patient population, it may take many years of marketing to sell enough drug to recover the investment in research and regulatory compliance.

S.2060 proposes a "cap of $ 200 million" in cumulative sales for a drug to maintain its orphan drug market protections. The problem with selecting an arbitrary number is that it may be too low for some drugs and too high for others. IL-2 is a good example.

The investment required to develop IL-2 and to get through the FDA approval process now exceeds $ 120 million--and it hasn't been marketed yet. More investment is needed to get the drug into the hands of doctors. Inventories have to be built. Capital expenditures may be needed to scale up production beyond the level of producing research quantities.

The capital requirements of bringing a drug like IL-2 to market could easily exceed the cash flow and profits generated by $ 200 million in cumulative sales. So, while $ 200 million may sound like a lot of money, it may not be enough to allow capital recovery. And if a company can't recover its investment, it has virtually no incentive to make the product.

We could, of course, try rate of return regulation rather than a sales cap. Rate of return regulation is based on allowing a company to earn a rate of return which is comparable to the rate enjoyed in other, equally risky businesses. This approach, which is widely used in public utility regulation, works fairly well so long as risk is measurable, highly predictable and the main use of capital is for plant and equipment.

However, in the public utility industry, rate of return regulation has failed when it comes to nuclear power, the high technology and long construction lead-time end of the utility business. Given the high investment risks of drug R&D, rate of return regulation would not work well because it would be impossibly difficult to measure risk and peg the rate of return to comparable investments.

TECHNOLOGY & HEALTH

Chiron's Drug for Kidney Cancer Heads For Approval, but Sales May Be Limited

By MARILYN CHASE
Staff Reporter of THE WALL STREET JOURNAL

Chiron Corp.'s new drug for advanced kidney cancer appears headed for Food and Drug Administration approval within months, but its high cost and severe toxicity may limit early sales to a narrow market.

Rebuffed 18 months ago, Interleukin-2 on Friday won the recommendation of an FDA advisory panel after a five-hour hearing. Studies indicate IL-2 offers a chance of complete remission for a few patients, yet it may hasten death for others—a risk which confines its use to patients able to bear its harsh and potentially lethal side effects.

Already approved in Europe at prices up to $9,000 per treatment, IL-2's final market clearance must come from the office of FDA Commissioner David Kessler. The commissioner isn't bound by panel recommendations, but they are typically given great weight.

Chiron shares, already buoyed by a market evidently expecting success, rose to $72 before settling down to close at $69, up 25 cents a share. Chiron acquired the IL-2 product last month when it bought the drug's developer, Cetus Corp., for stock valued at $650 million. Both biotechnology companies are based in Emeryville, Calif.

"We're obviously pleased," said Chiron President Edward Penhoet. "We expected this result, but you can never be sure."

Chiron's marketing plans for IL-2 include intensive training on how to mute the drug's side effects, and boost a patient's chances of benefit.

Current European sales of IL-2 run $15 million a year, and he forecast that its initial U.S. market could double that. IL-2 is a genetically engineered copy of a natural human protein which boosts the numbers and the tumor-fighting power of white blood cells.

IL-2, not yet priced in the U.S., sells in Europe for $7,000 to $9,000 per course of treatment. Because patients must take IL-2 under the close monitoring of an intensive care unit, the cost of hospitalization will boost total cost to $30,000 to $45,000 per treatment. Patients who respond well to IL-2 often take a second or even a third course of the drug—doubling or tripling the ultimate bill.

For some 20,000 patients newly diagnosed with kidney cancer each year in the U.S., IL-2 represents a weapon that is distinctly double-edged: high risk but also potentially high reward. About 60% of kidney cancer metastasizes or spreads beyond its original site, leaving patients with no cure and a dismal prognosis of six to 12 months. Studies presented at Friday's hearing indicate those odds are starting to improve.

Of 255 patients studied, Chiron researchers said at the hearing that IL-2 made tumors shrink or disappear in 37 patients, or 15%. That included nine patients, or 4%, who went into complete remission. The good responses have lasted nearly two years on average, and eight of the nine patients in complete remission remain cancer-free. But 11 patients died from side effects of treatment, mostly lethal heart and lung problems provoked by the stress of massive fluid retention in the body.

"Approval of IL-2 represents an important step in the development of immunotherapy," said Eugene Schonfeld, president of the National Kidney Cancer Association. Mr. Schonfeld had lectured the FDA panel on its "moral obligation" to approve IL-2. He held the panel partly accountable for some 15,750 kidney cancer deaths that occurred since it declined to sanction sale of the drug back in June 1990.

But the cool scholarly presentation by Chiron was much more persuasive than Cetus's heated and impetuous bid for approval 18 months ago. FDA panel chairman Jerome Groopman of Harvard University this time called the clinical responses "impressive and [not] anecdotal." Even though the drug has yet to lengthen lifespans for most patients, it offers vivid hope for a few. Pioneering IL-2 researcher Steven Rosenberg of the National Cancer Institute documented some graphic recoveries with slides of bulky tumors melting away to a shadow or less.

It wasn't enough for the lone dissenter in Friday's 7-1 vote. "I remain unconvinced," said Ernest Borden of the Medical College of Wisconsin. He noted the drug's difficult risk-benefit ratio: "twice as many patients suffered severe neurological toxicity . . . as those who responded favorably."

"IL-2 isn't a cure," said National Cancer Institute Director Samuel Broder in an interview. Still, he noted, winning rare and difficult remissions among patients once deemed incurable, is an important step. The FDA panel's vote validates a decade of federally sponsored research, he added.

Dr. Penhoet confirmed Chiron's intent to build on IL-2's initial niche, expanding it into new uses for other cancers and infectious diseases, such as acquired immune deficiency syndrome. IL-2 is currently being tested in melanoma and AIDS, and in combination with a variety of tumor-fighting cells, though the executive didn't forecast how soon Chiron might seek the FDA's nod.

Now for some economic data. The following table shows average R&D to sales ratios, average annual growth rates for R&D investment and net sales for all publicly owned companies within each industry sector which produces drugs. These data are from R&D RATIOS & BUDGETS, 1991 Edition, published by Schonfeld & Associates. These data are compiled from the audited financial statements which companies file with the SEC in 10-K reports.

SIC	Industry Sector	Average R&D as % Sales	% Growth in R&D $	% Growth in Sales
2833	Medicinal chemicals	10.2	12.2	12.8
2834	Pharmaceuticals	9.6	14.0	12.9
2835	In vitro/in vivio diagnostics	11.0	14.1	14.0
2836	Biological products	12.6	15.1	19.6
8731	Biotechnology research	21.4	13.4	15.2
3841	Surgical/medical instruments	5.5	13.0	11.2
3845	Electromedical apparatus excluding X-ray machines	9.7	11.3	10.3

High rates of R&D investment in these industry sectors are producing good results as shown by growing sales, encouraging more R&D investment as shown by the growth rates for R&D.

Comparing data for the drug industry with data for other, large, well known companies, it is clear that the drug companies are investing more R&D as a percentage of sales than even the best managed U.S. industrial companies such as GE, Hewlett-Packard, IBM and Motorola--and much more than GM and Ford. The drug companies are also producing sales growth and reinvesting in R&D at an increasing rate.

SIC	Company	Average R&D as % Sales	% Growth in R&D $	% Growth in Sales
3711	Ford Motor Company	3.9	9.0	2.8
3711	General Motors	4.6	6.8	.9
3600	General Electric	2.5	6.6	7.0
3570	Hewlett-Packard	10.3	11.1	11.8
3570	IBM	7.4	8.5	6.5
3663	Motorola	9.5	14.2	11.5

As every American knows, General Motors is no longer a competitive company and the American automobile industry is stalled. These data show part of the reason why GM is no longer a competitive company.

Sometimes people think profit is a dirty word. But profit is what sustains R&D programs and risk taking. I do not fault drug companies for making money.

By reducing the rate of return on orphan drugs, or by removing market protections for orphan drugs, or by reducing orphan drug incentives, Congress will reduce the rate of investment in orphan drug development.

Congress must remember that R&D capital is the highest form of risk capital. When a company buys a piece of machinery, it can always sell it and recover part of its investment if things don't work out. In the world of R&D, if the research doesn't pan out, the money is gone.

In an R&D intensive industry, as measured by R&D to sales, companies must get high rates of return to compensate for the capital which is put at risk. Therefore, I do not fault the drug companies for making good profits. In fact, if the drug industry were more profitable, it would attract even more investment, and develop even more drugs.

In considering drug industry profits, it is important to look at the data. My company publishes FINANCIAL BASELINE FORECASTS which contains financial data on every publicly owned company with annual sales over $ 10 million. In industry sectors 2833-2836, for calendar year 1992, I compiled the following list of 71 drug companies showing:

net income in thousands of dollars
% return on assets = net income / total assets
% return on equity = net income / book value of equity
% growth in sales
% growth in net income

COMPANY NAME	NET INC	% ROA	% ROE	%GSALES	%GNETINC
BRISTOL MYERS SQUIBB	2335213	23.88	39.70	15.76	20.53
MERCK & CO	2265737	24.02	47.69	8.25	13.51
LILLY (ELI) & CO	1340516	16.48	26.26	6.33	15.89
JOHNSON & JOHNSON	1291702	11.69	21.95	11.87	2.92
AMERICAN HOME PRODUCTS CORP	1251419	20.04	37.68	7.77	4.47
ABBOTT LABORATORIES	1159898	17.77	32.61	8.48	10.08
MARION MERRELL DOW INC	992014	38.62	62.08	49.58	79.39
PFIZER INC	880561	9.66	17.58	4.12	0.52
SCHERING-PLOUGH	686029	15.36	32.15	4.06	13.77
WARNER-LAMBERT CO	596439	16.41	36.51	7.43	12.46
UPJOHN CO	533331	13.16	28.11	3.68	6.86
BAXTER INTERNATIONAL INC	529816	5.98	12.68	7.80	5.55
SYNTEX CORP	485116	19.09	183.90	14.13	12.58
RHONE-POULENC RORER	221071	5.62	29.33	-68.70	8.01
AMGEN INC	130888	6.18	6.65	88.42	82.32
IMCERA GROUP INC	100515	5.03	11.65	7.22	-6.84
ALLERGAN INC	87718	9.21	14.88	3.80	8.07
ALZA CORP -CL A	54832	9.30	14.04	35.19	68.69

COMPANY NAME	NET INC	% ROA	% ROE	%GSALES	%GNETINC
CARTER-WALLACE INC	49850	8.00	11.25	8.08	10.38
FOREST LABORATORIES -CL A	48785	11.18	13.28	27.29	15.11
GENENTECH INC -RED	40066	3.33	4.26	25.82	-34.70
MYLAN LABORATORIES	25589	11.92	12.84	3.37	-26.59
DIAGNOSTIC PRODUCTS CORP	22742	17.71	20.74	13.21	17.09
SPI PHARMACEUTICALS INC	20607	9.80	12.76	24.29	14.27
A.L. LABORATORIES INC -CL A	18922	5.72	16.53	6.91	25.23
BIOGEN INC	14103	8.89	9.48	31.89	61.02
LIFE TECHNOLOGIES INC	13205	10.30	13.09	8.19	-1.87
BIOCRAFT LABORATORIES INC	12409	6.44	10.12	13.16	728.96
MED CHEM PRODUCTS INC	7406	17.51	19.09	-41.29	42.27
E-Z-EM-INC	6562	8.65	11.15	5.90	112.87
MOLECULON INC	6521	18.08	28.60	3.08	14.92
IMMUCOR INC	5460	14.68	17.33	83.13	65.22
CHATTEM INC	5417	9.05	39.81	13.54	27.68
ZENITH LABORATORIES	5288	15.05	23.10	-20.98	92.24
LEINER (P) NUTRITIONAL PRODS	4911	6.20	10.70	-44.05	7.55
GENZYME CORP	4774	2.76	3.15	29.44	-20.54
NATURES SUNSHINE PRODS INC	4540	16.63	22.05	16.80	1.24
IVAX CORP	3765	2.90	4.07	30.85	-42.56
JONES MEDICAL INDS INC	3735	10.06	11.03	21.62	36.53
QUIDEL CORP	2685	33.12	266.68	773.69	485.20
IDEXX LABS INC	2005	10.29	13.81	24.75	-20.12
HAUSER CHEM RESH INC	1794	16.28	23.33	-100.00	70.05
MOLECULAR BIOSYSTEMS INC	1758	2.98	3.62	11.81	32.41
MARSAM PHARMACEUTICALS INC	1444	6.06	6.44	5.26	134.48
TECHNE CORP	1400	12.85	19.31	21.11	14.32
HALSEY DRUG CO INC	1165	4.51	9.14	26.93	-27.13
BARR LABORATORIES INC	919	1.03	2.07	8.13	-82.40
MERIDIAN DIAGNOSTICS INC	913	8.88	9.85	30.92	-4.72
HYCOR BIOMEDICAL INC	909	7.64	8.57	6.56	-0.33
GAMMA BIOLOGICALS INC	785	5.20	5.55	-2.76	-12.19
NATURE'S BOUNTY INC	781	1.67	6.36	12.71	2.40
NORTH AMERICAN BIOLOGICALS	603	2.32	4.29	-30.65	-72.67
NATURAL ALTERNATIVES	598	7.57	13.20	21.16	45.92
ARMSTRONG PHARMACEUTICALS	428	4.22	5.96	1.41	-72.53
ADVANCED MAGNETICS INC	239	0.66	0.69	30.68	21.49
IGI INC	108	0.55	1.23	9.21	-43.29
INCSTAR CORP	-268	-0.53	-0.94	27.46	-107.84
CARRINGTON LABS	-730	-8.40	-20.41	38.39	-14.60
K V PHARMACEUTICAL -CL B	-1279	-3.56	-8.29	3.19	-106.72
LEECO DIAGNOSTICS INC	-2809	-10.55	-20.87	9.70	2.86
VESTAR INC	-3773	-23.87	-48.34	21.28	25.26
PHARMACONTROL CORP	-4351	-25.99	0.00	-0.36	8.83
CAMBRIDGE BIOTECH CP	-6781	-16.66	-25.49	10.40	-91.25

COMPANY NAME	NET INC	% ROA	% ROE	%GSALES	%GNETINC
SCHERER (R.P.)/DE	-9620	-1.79	-83.39	-3.95	-380.52
CENTOCOR INC	-11093	-2.36	-3.59	-14.61	87.48
IMMUNEX CORP	-14137	-7.96	-77.25	13.20	-98.37
NOVA PHARMACEUTICAL CORP	-16310	-37.84	-54.24	32.58	-9.19
COLUMBIA LABORATORIES INC	-16358	-127.28	0.00	33.68	-41.09
PHARMACEUTICAL RES INC	-21258	-24.07	-42.13	116.72	-50.50
ICN PHARMACEUTICALS INC-DEL	-42114	-11.88	0.00	25.67	N/A
CETUS CORP	-100233	-37.14	-118.36	31.47	-27.00

This table shows several things. First, there are approximately 175 publicly owned companies in the industry which file documents with the SEC. Over half of them didn't get included in the list because they were too small with sales under $ 10 million. Many drug companies do not have the capital required to generate $ 200 million in cumulative sales, the suggested sales cap in S.2060.

Second, some very large companies earn handsome profits and enjoy an excellent return on total assets, the amount of capital employed. Many of these companies are large, diversified companies with substantial consumer products businesses in addition to their drug businesses. Clearly economies of scale are at work. If orphan drug sales are capped, they can invest elsewhere.

Third, only 25 of the 71 companies, earn a double digit ROA or rate of return on capital of more than 10 percent. 21 percent of the 71 firms, are forecast to generate losses, not profits. If the small drug companies with sales under $ 10 million were included, many more companies would be shown as unprofitable.

When we look at biotechnology companies, we see that many barely make it at all. The most profitable is Chiron which is projected to generate annual net income of only $ 10.7 million and a return on total assets of 2.88 percent, far less than a pass book savings account. Most biotech companies didn't make the list at all because they have sales less than $ 10 million and heavy losses.

COMPANY NAME	NET INC	% ROA	% ROE	%GSALES	%GNETINC
CHIRON CORP	10685	2.88	4.91	12.22	-9.40
KRUG INTERNATIONAL CORP	7776	25.87	N/A	-8.38	545.92
MAXWELL LABORATORIES INC	2800	4.98	7.43	7.29	-4.87
INTL RESEARCH & DEV CORP	1168	2.01	4.01	11.16	12.25
THERMO ELECTRON TECHNOLOGIES	374	0.00	0.00	-9.09	49.34
KMS INDUSTRIES INC	228	1.94	2.33	-9.69	N/A
SPIRE CORP	208	1.29	2.21	-24.24	203.98
ENERGY CONVERSION DEV	-338	-3.51	0.00	-18.96	-228.46
AURA SYSTEMS INC	-800	-3.72	-4.43	63.14	61.86
TSI CORP	-923	-2.33	-2.95	8.60	-127.39
MYCOGEN CORP	-4872	-5.34	-5.59	13.54	-49.41
GENETICS INSTITUTE INC	-23094	-11.67	-13.14	-13.02	-25.06
XOMA CORP	-29509	-29.41	-31.58	19.10	-2.76

In summary, the drug industry has a few very profitable, large companies. However, many companies, particularly small ones, have not yet reached a size where they enjoy substantial profits.

Further, it is important for Congress to understand just how dependent the American people have become on private industry R&D as opposed to Federal R&D as a source of new drugs and healthcare innovations. Several months ago, I gave a presentation on Capital Hill which examined Federal spending for healthcare research, and cancer research in particular.

The accompanying graphs, Figures 8 and 9, show that in healthcare, industry R&D spending has surpassed Federal R&D spending.

Figure 11 shows trends in R&D as a percent of net sales for 7 leading drug companies and compares them to Federal healthcare R&D spending as a percent of U.S. healthcare costs. These graphs clearly show that for ten years, private sector R&D has been making up for the slow growth of Federal R&D.

Figure 12 compares the R&D budgets of IBM and General Motors with the budget of the National Cancer Institute. R&D growth at these two firms is much faster than for the National Cancer Institute, and both GM and IBM are going through tough times. The Federal government is clearly under-funding research for cancer, the nation's most costly disease.

Figure 14 shows that in constant 1980 dollars, Federal spending for cancer research actually declined from $ 1,271 per new case in 1980 to $ 793 per new case in 1991, on an inflation adjusted basis. As the nation's most costly disease, the U.S. is not controlling the cost of cancer in the U.S. economy.

Keep in mind that many forms of cancer are orphan diseases. There are over 200 forms of cancer, each with its own biological properties. Therefore, each requires different drugs, diagnostic tests, and care.

I don't mean to be an apologist for the drug industry, and I have no investments in the drug or healthcare industries. I just understand the laws of economics, which many people in Washington seem to ignore.

The Orphan Drug Act has made investment in R&D for orphan diseases more attractive and has stimulated many new drugs for these diseases. I believe this was the intent of the law and it is working.

Congress deserves a pat on the back. Congress helped many people with dreadful diseases. Take credit for the success. Learn to be a gracious winner.

In general, I value the time of Congress. But too often, Congress spends its time worrying about the wrong things. Trying to "fine tune" the Orphan Drug Act offers very little pay off for the American people because the Act is working. It doesn't need fixing.

U.S. SPENDING FOR HEALTHCARE R&D

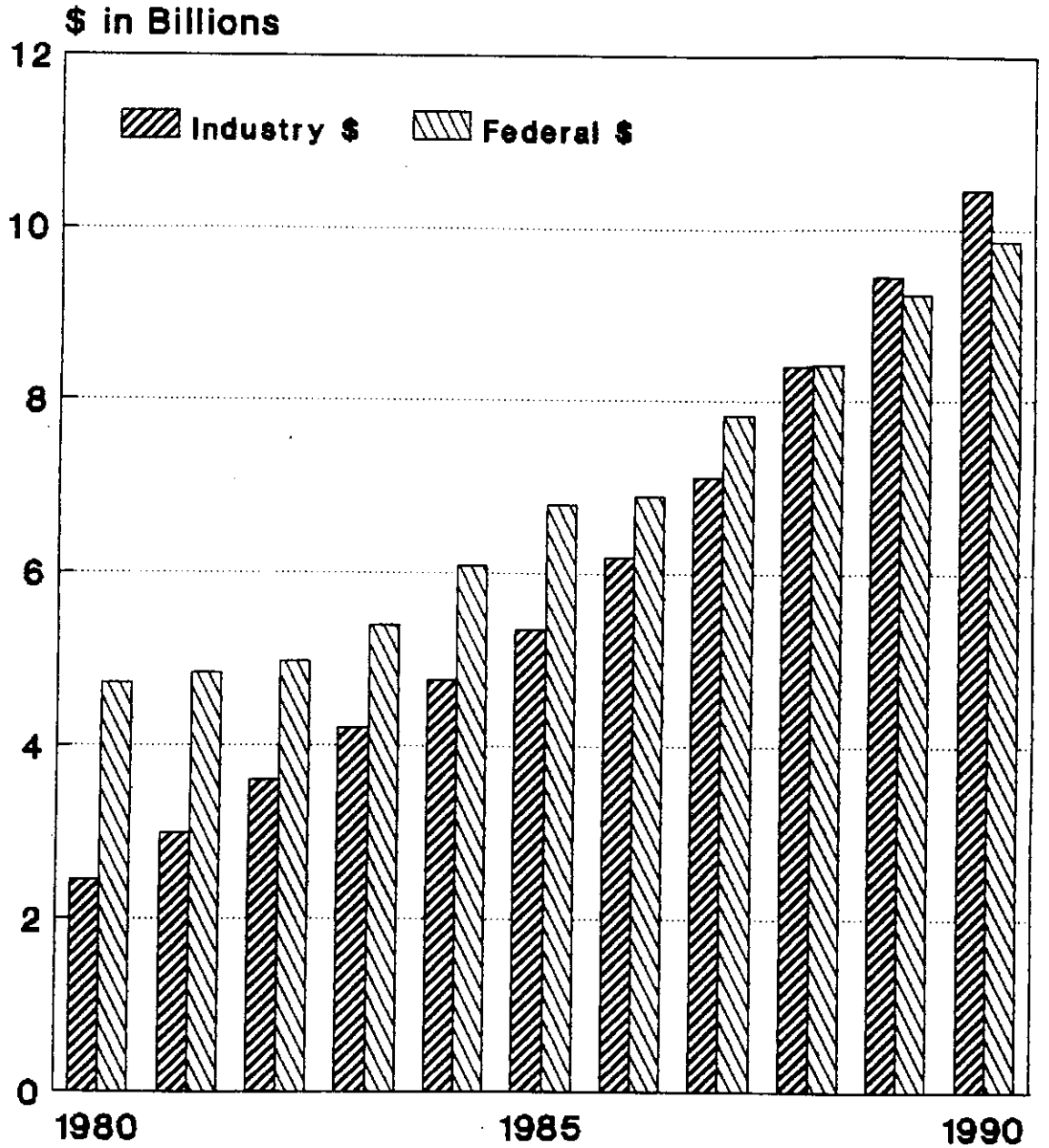

Fig. 8: NIH Data Book, 1990

% SHARE U.S. HEALTH R&D

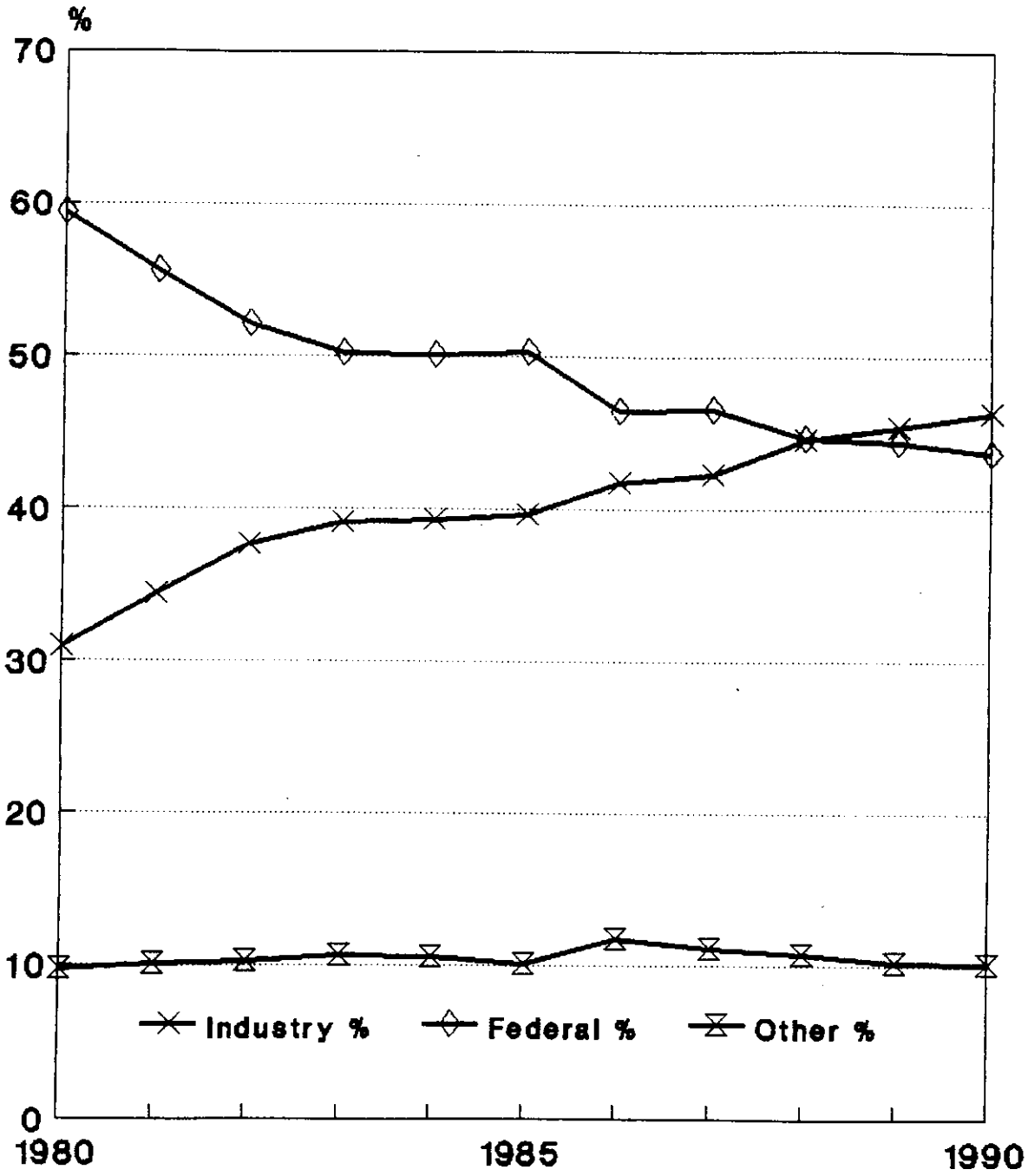

Fig 9: NIH Data Book, 1990

R&D AS % SALES 7 DRUG COS. vs. FEDERAL R&D AS % COSTS

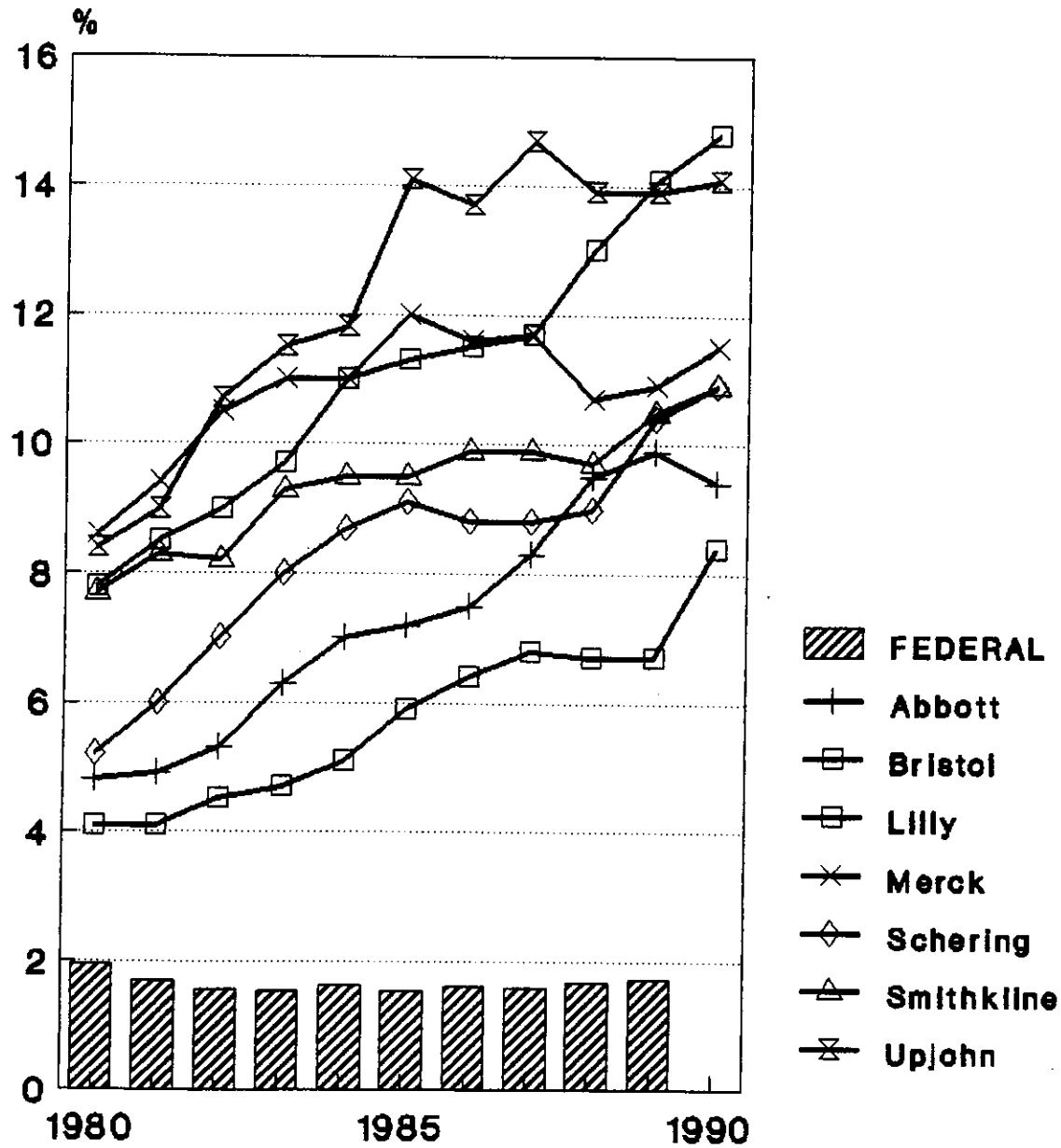

Fig. 11: R&D Ratios & Budgets
NIH Data Book, 1990

NCI's BUDGET vs. R&D $'s of GM and IBM

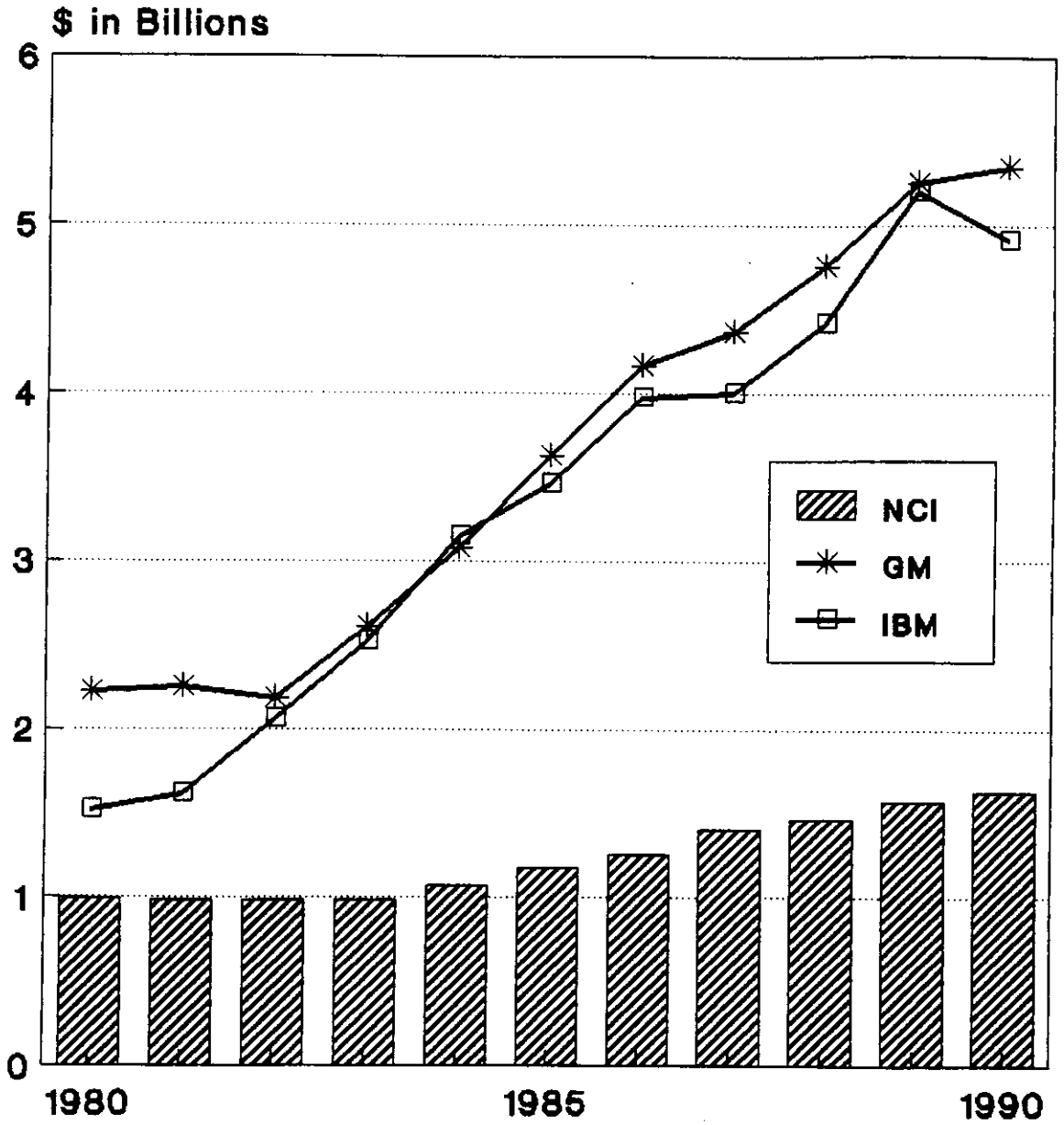

Fig. 12: R&D Ratios & Budgets
NIH Data Book, 1990

FEDERAL SPENDING PER NEW CANCER CASE

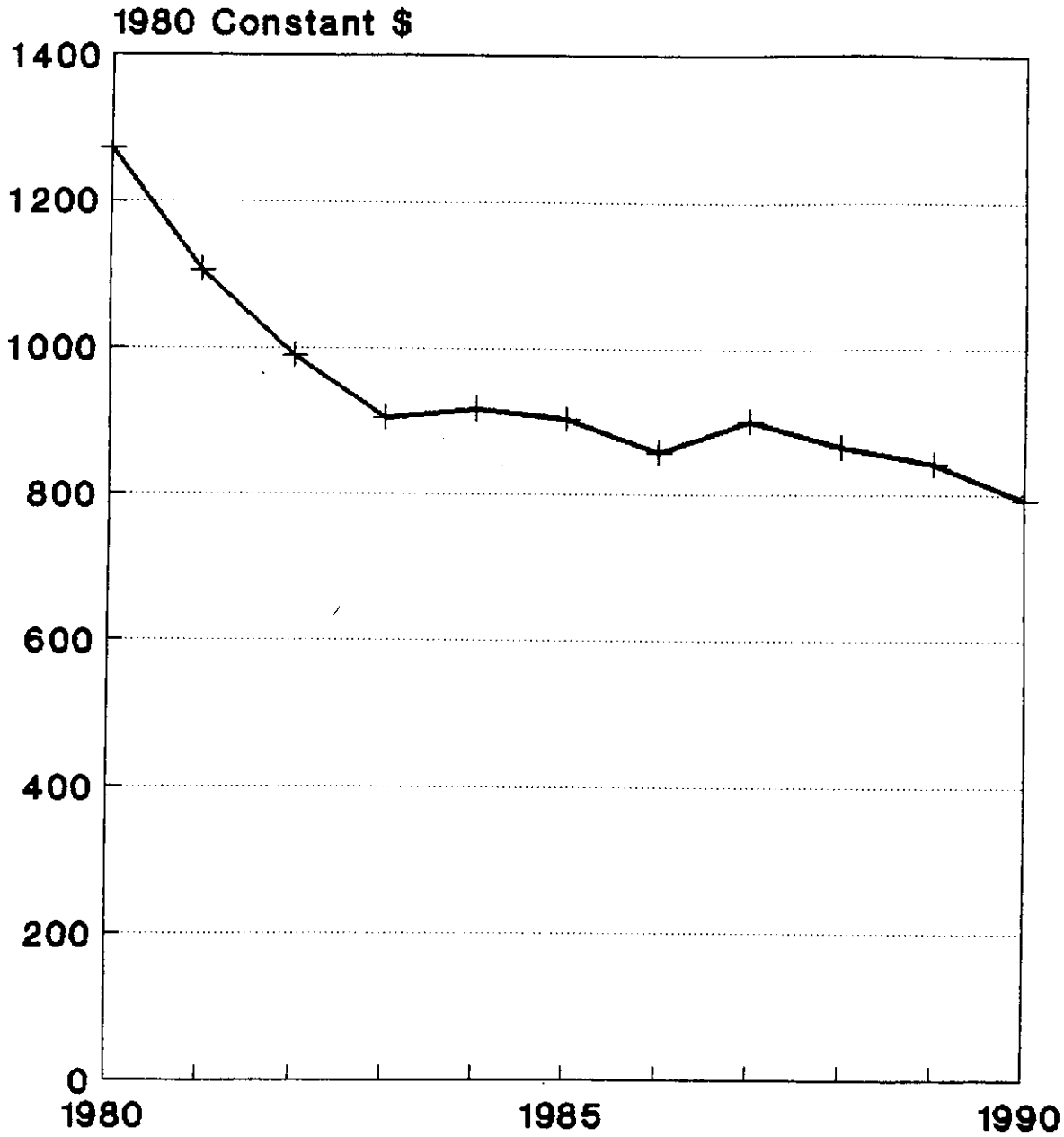

Fig. 14: NIH Data Book, 1990; ACS, 1991

If you insist on "fine tuning," I recommend that you decrease the cost of Federal regulation for orphan drugs--not by compromising efficacy and safety--but streamlining the FDA approval process. The time required to get new drugs through the process is literally killing people. During the eighteen month delay on IL-2, 15,750 kidney cancer patients died. Many of them could have been helped if the FDA process had worked more swiftly.

The FDA must consider the consequences of its actions. Efficacy and safety are important to be sure, but so is the time and the cost of the regulatory process. The cost of FDA regulation is passed on to patients, to insurance companies, to employers and to American taxpayers through government paid care.

In fact, the American people are paying twice for the FDA. We pay its operating budget, but we also pay the hidden cost of regulation which is built into drug prices. I urge you and others in Congress to consider increasing the FDA's budget so it can attract more qualified people, streamline its operations with computers and other technology, and do a better job.

The Orphan Drug Act, growth of the biotechnology industry, and expanding drug company R&D budgets are important and beneficial forces spurring innovation and new drugs. All drugs must pass through the FDA. However, the budget of the FDA has not kept pace with the growth of industry R&D--which is why the FDA is currently sitting on a backlog of over 8,000 drug applications. Surely, some of these drugs could save lives and reduce the cost of care for U.S. taxpayers.

Rather than worry about orphan drug prices and markets, your time and the time of the Committee would be better spent on FDA reform, on increasing Federal healthcare R&D programs, and on reforming healthcare insurance to squeeze out administrative waste and cost.

Look to where you can get the greatest leverage on controlling healthcare costs because you'll reduce the cost of orphan disease care as well as the cost of all diseases. For example, to really save money, launch a program to educate the American public on how to shop for healthcare services. The most expensive care is "ineffective" care which costs lives and forces patients into extended care situations. Smart shopping would really manage costs.

Many well intentioned people may express views contrary to those in this letter. Just ask yourself how much they know about economics and R&D. Thank you for the Orphan Drug Act. Please have the good sense to leave it alone. Enjoy your success and start working on other problems in healthcare.

Sincerely,

Eugene P. Schonfeld, Ph.D.
President

cc: Senator Orrin G. Hatch