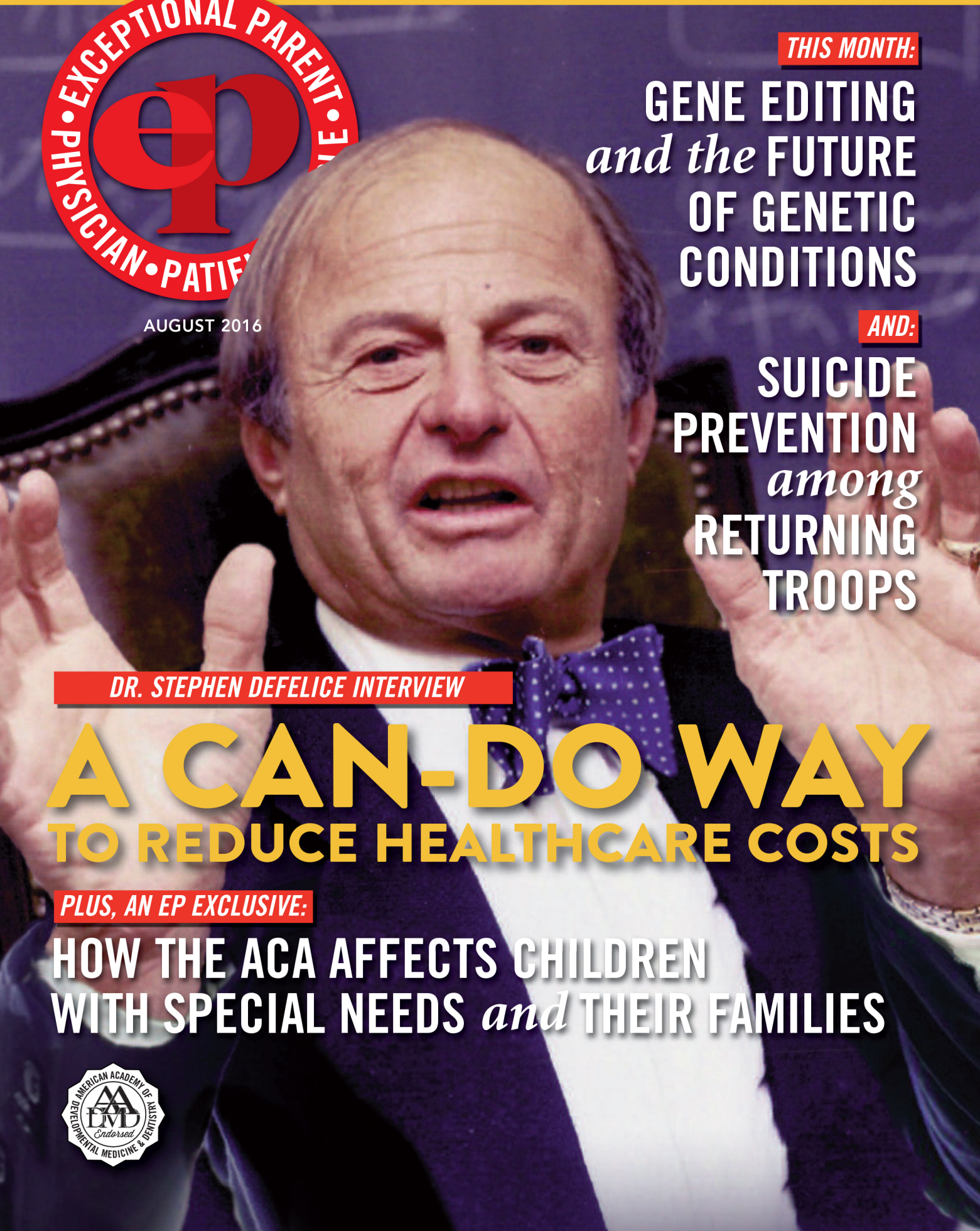
PLUS: UNITED STATES MILITARY SECTION



EXCEPTIONAL PARENT • PHYSICIAN • PATIENT

AUGUST 2016

THIS MONTH:

GENE EDITING *and the* FUTURE OF GENETIC CONDITIONS

AND:

SUICIDE PREVENTION *among* RETURNING TROOPS

DR. STEPHEN DEFELICE INTERVIEW

A CAN-DO WAY TO REDUCE HEALTHCARE COSTS

PLUS, AN EP EXCLUSIVE:

HOW THE ACA AFFECTS CHILDREN WITH SPECIAL NEEDS *and* THEIR FAMILIES

AMERICAN ACADEMY OF DEVELOPMENTAL MEDICINE & DENTISTRY — AADMD Endorsed

PART I *of a* SERIES

PROMINENT PHYSICIAN PROPOSES A CAN-DO WAY TO REDUCE HEALTH CARE COSTS – *BY FINDING CURES*

INTERVIEW OF STEPHEN L. DEFELICE, M.D., BY JOSEPH M. VALENZANO JR.

There is general agreement that the US health care system is in crisis with no doable solution in sight. The ever-increasing cost of health care is the biggest concern of policymakers, politicians and the American people. Access to health care is also of great concern to millions of Americans who have health insurance that, ironically, they cannot afford to use. The controversial Affordable Care Act has already substantially raised health care costs with no end in sight.

Into this chaotic situation steps "A Man with a Plan," Stephen L. DeFelice, the founder of the Foundation for Innovation in Medicine (FIM), a physician with a long, creative career in medicine. He approaches the crisis in health care in a radically different manner. He is not introducing new policies or bureaucratic programs, but instead, a practical program of action, to be carried out by brave women and men he calls "Doctornauts."

What is a Doctornaut? Simply put, *it is a physician-patient who will volunteer for clinical research of pharmaceuticals, natural substances or new medical devices under the supervision of a physician-clinical researcher with minimal FDA, institutional or other restraints.*

Dr. DeFelice has outlined this approach in "The Doctornaut Act," a discussion draft of which was circulated by Senator Bill Frist and available on the FIM website (www.fimdefelice.org).

What will most effectively bring down health care costs? The answer, according to Dr. DeFelice, is finding cures. Who can argue? Cure diabetes and there will be no costs.

What is needed to find cures? Clinical trials of promising new treatments, which now face extraordinary obstacles: New therapies, however, can only be discovered in clinical trials.

What is his definition of cures? A cure is any therapy that either prevents or eliminates disease or disabilities by treatment.

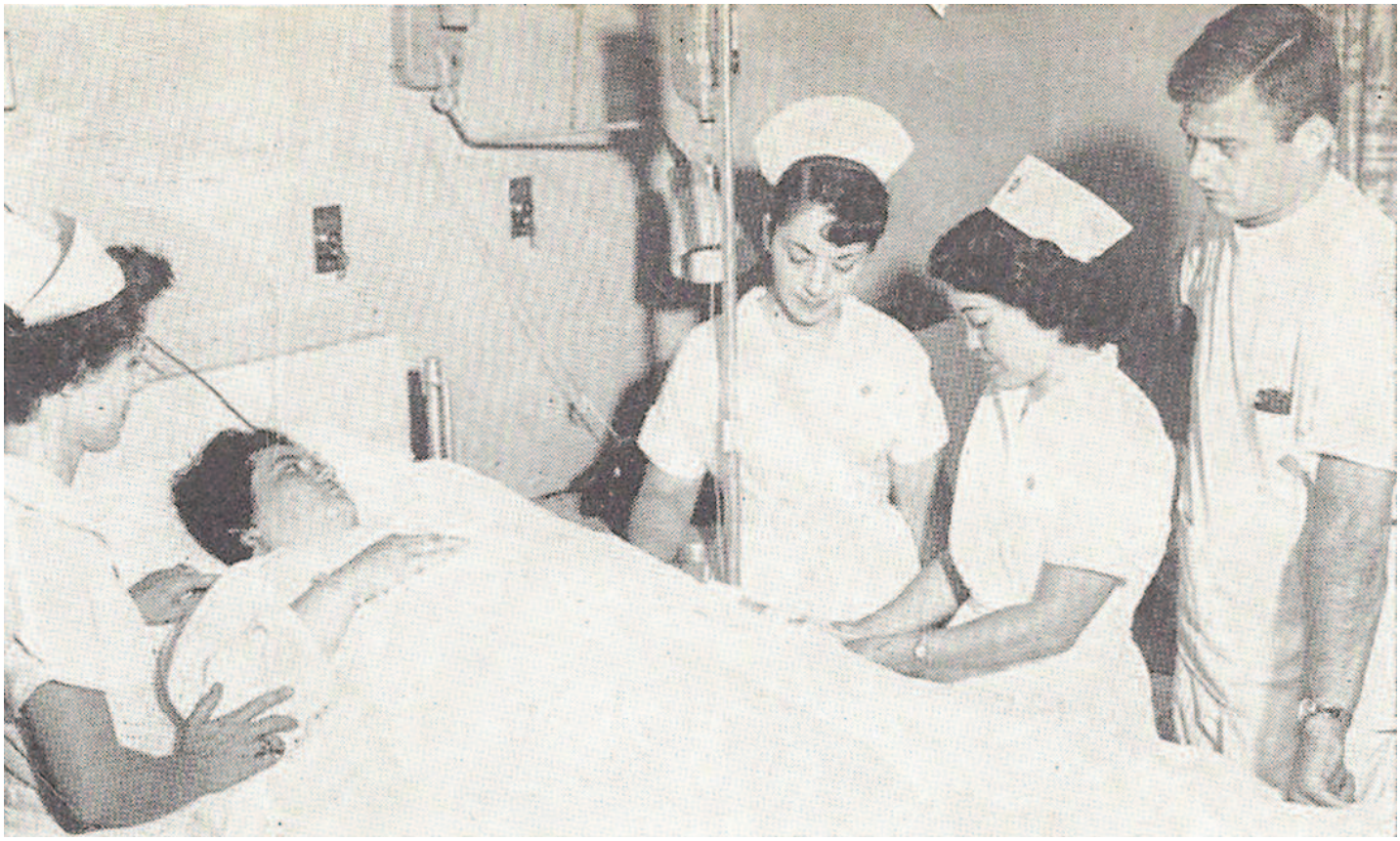
What then can overcome the obstacles in the way to the vitally necessary clinical trials? The Doctornaut Act, which will allow the clinical testing of promising new drugs, natural remedies and medical devices quickly and bring about the new cures that are needed.

The ancient Greeks had their Argonauts who sailed unknown seas on dangerous journeys. The Russians had Cosmonauts and the Americans had Astronauts who sailed space craft on dangerous journeys into the cosmic ocean. All of them took great risks to advance knowledge and improve the life of mankind. Some of them suffered – even died – in this effort. They are considered heroes for their bravery. Dr. DeFelice suggests we need a new breed of heroes, this time in medicine – Doctornauts who will bravely and altruistically head into uncharted medical waters in search of cures in the short rather than the long term.

While the term Doctornaut may be new, the concept is part of a long tradition in medicine in which physicians have practiced self-experimentation, trying out new and risky treatments on themselves first. A century ago, Werner Forssmann, a German physician, inserted a catheter in his vein and guided it to his heart. This risky act revolutionized the field of cardiology and he was awarded a Nobel Prize. More recently, Australian physician Barry Marshall swallowed a concentrated solution of H. *pylori* to prove his theory that this bacterium causes gastrointestinal ulcers and gastritis. His brave act was a major medical breakthrough for which he also was awarded a Nobel Prize. History is replete with self-experimenting courageous doctors in the search for cures. In this process, error and harm are unavoidable, even the possibility of death.

Dr. DeFelice himself is in this tradition. As a young doctor, working with physicians and nurses in Yugoslavia, he acted as a true

PHOTO PROVIDED BY STEPHEN L. DEFELICE, MD FROM THE NEW YORK TIMES



TO BOLDLY GO: An early photo shows Dr. Stephen L. DeFelice *(far right)* working with with a team of nurses during an intravenous procedure. He suggests that we need Doctornauts, a new breed of heroes, who will bravely and altruistically head into uncharted medical waters in search of cures in the short rather than the long term.

Doctornaut to move one of his studies forward. He injected himself with two different carnitine solutions in separate arm veins to test for safety. The results permitted him to proceed with other foreign and U.S. clinical studies which were instrumental in obtaining FDA approval for carnitine that saves the lives of thousands of children, both in our country and abroad.

Today, our risk-averse culture is not willing to take such chances. That is why Dr. DeFelice is working so hard to make the Doctornaut Act a reality. He is convinced that history and current trends indicate that physicians will step up to the plate and take the risks others fear to take or which our cultural rules prohibit. In order to avoid the misconception that we are dealing with doctors gone wild, Dr. DeFelice emphasizes that the vast majority of such clinical studies will not be life-threatening because physicians understand, better than others, what the benefits/risks are.

Dr. DeFelice is doing his best to see that this actually occurs through Doctornauts and the approach he calls "Cure Care vs. Health Care" and how they are related.

If President Obama can undertake an ambitious $1 billion "Cancer Moonshot" to eliminate cancer in his last year in office, perhaps the next President can start out by supporting the Doctornaut Act which will deal with all diseases. This will increase innovation in medicine and accelerate the discovery of cures for the costly major diseases that plague humanity, including diseases and disabilities in children. "Cure Care" will deliver those treatments to the American people – soon.

> The Doctornaut Act will increase innovation in medicine and accelerate the discovery of cures for major diseases that plague humanity, including diseases and disabilities in children.

I first met Dr. DeFelice in the 1980s at a FIM conference. I was intrigued by his straightforward, no–nonsense message: the best way to reduce health care costs is by curing disabilities and disease by prevention and treatment.

But what intrigued me even more was how he proposed to discover these cures. Years ago, he proposed that Congress pass the Doctornaut Act. He had support from Senator Bill Frist, a physician and then Senate Majority Leader. It's based on the premise that the only way to discover new therapies is to test them in clinical studies in patients. For example, penicillin could not be discovered until tested in patients with bacterial infections, and insulin in diabetic ones. There is indisputable, published evidence of the enormous obstacles to clinical testing of new therapies. Dr. DeFelice calls this the Barrier System in which large numbers of promising therapies have not been and never will be tested. For this reason, the discovery of

cures is a rarity despite our exploding technology of which our culture doesn't get the connection.

Few appreciate the enormous sums of money – billions upon billions of dollars – spent on research on diseases, such as cancer, cardiovascular, mental, neurological, arthritic and pulmonary and many others, without the discovery of cures. The NIH annual budget for medical research is approximately 32 billion dollars. Over the past decade the NIH, apart from the pharmaceutical industry, has funded close to 50 billion dollars on cancer research without the discovery of major cures. There are close to three million patients with the primary diagnosis of epilepsy, with 32 drugs available as treatment, none of which is a cure. Patients over the age of 65 take a daily average of five drugs, none of which is a cure.

Oftentimes, less prevalent conditions, including disabilities such as Down's syndrome, escape sufficient national attention and how our aging population is changing the status quo of the disabled and their families. In the past, these children left us in their twenties, but due to modern therapies they can now live up to the age of 60, when their parents, however, are much older and afflicted with the costly chronic diseases of aging. So we are dealing with the long-term suffering of two very costly and suffering patient populations in a single household without the availability of cures – an unacceptable outcome, if there ever was one.

The no-cure list is long. Dr. DeFelice has unsuccessfully attempted to have our country ask the challenging question, "Why are there so few cures?"

The Doctornaut Act will rapidly overcome the barriers which block the discovery of cures as well as more effective therapies. It will permit physician volunteers to freely volunteer for early clinical trials, some risky and, importantly, waive their right to sue. *If enacted, the base of medical innovators would immediately broaden; more promising therapies would be tested; more medical discoveries would reach patients, curing many. Because of his experience, he also believes doctornauts would immensely benefit children. And these benefits would occur in the short-term.*

Despite decades trying to convince Congress to pass the Doctornaut Act, he has, with the exception of Senator Frist, repeatedly run into a stone wall. But he's betting that the current presidential race will produce an opening for his innovative ideas.

Dr. DeFelice believes the next president could seize the moment and help accelerate the discovery not only of cures but also of low cost medical breakthroughs through the Doctornaut Act. He plans to deliver his message of Cure Care versus Health Care to the candidates during the presidential race.

> The Doctornaut Act will overcome barriers that block the discovery of cures, permitting physicians to volunteer for early clinical trials and, importantly, waive their right to sue.

When I asked what sparked his passion to pursue the passage of the Doctornaut Act, DeFelice attributed it to three personal experiences: his grandmother's diabetic coma; a child with leukemia; and his discovery and pursuit of the natural substance, carnitine – an interesting triad, to say the least.

When he was 12, his grandmother, or "nonna," was in diabetic coma lying on a bed in the dining room without hope of recovery. There was a 24-hour vigil by family and friends. He couldn't accept the fact that she would die and he talked to her, trying to elicit some type of response, which failed. He then went to the local Catholic Church and made a deal with God promising to do good things if He saved her life. He was convinced he had made a deal. But she died that night.

He unexpectedly felt two powerful emotions: an intense hatred of disease and a strong conviction that disease must and can be conquered. He met only one person, 'Doc' Druckenmiller, a country doctor who he made rounds with when he was a medical student – $3 an office visit and $5 a house call – who proclaimed hatred for disease. About 15 years later, as a third-year medical student covering the pediatric ward, Dr. DeFelice cared for a nine year-old child with terminal leukemia. The mother and father were kneeling by her bed silently praying. He said, "The scene of Christ and the manger came to mind. The first scene dealt with life; the one before me with death. About an hour later, when I was alone with her, she expired. It hit me hard. One moment she was alive, the next gone forever. Incomprehensible!"

Only a handful of people know that it was Dr. DeFelice who brought carnitine to America in 1965. He conducted the first successful clinical studies on it. After repeated failures, he found funding for development through his friend, the late Claudio Cavazza, proprietor of Sigma-Tau Pharmaceuticals.

Together, they guided its way to FDA approval for the treatment of the fatal disease in children, Carnitine Deficiency, and also for patients on renal dialysis. It's also given to premature babies who fail to thrive and other conditions. His unparalleled experience in all sectors of clinical research qualifies him to be considered one of the world's top experts.

As we discussed his third experience, with carnitine, his adrenalin production skyrocketed. He began, "Carnitine taught me about the entire Barrier System which begins with the identification of the drug itself to FDA approval and beyond. If you understood the entire Barrier System, you would conclude that it was devised by a sadist who finds happiness by creating obstacles to keep promising medical therapy from being clinically tested and reaching physicians and patients."

In his first book, *Drug Discovery, the Pending Crisis,* published in 1972, Dr. DeFelice predicted, "Our present system of drug discovery is almost designed not to cure the great diseases that confront us. There is no doubt that many will be cured in the distant future, but it is unfortunate that many must wait." In this book, he first proposed physician volunteers or doctornauts for clinical studies as the solution.

According to Dr. DeFelice, the complicated Barrier System includes the nature of the drug, patents, funding, patient availability, doctors, universities, hospital Institutional Review Boards (IRBs), the FDA, the National Institutes of Health (NIH), the

pharmaceutical and medical device industries and many other factors. But the cultural mindset is the governor of the other components of the aforementioned. Interestingly enough, he knows of no one who has traveled through the entire system.

"How would you describe this cultural mindset?" I asked.

"It's a syndrome characterized principally by fear combined with ignorance, apathy and the absence of knowledgeable leaders who represent the patient. It's simply too difficult and costly to conduct clinical studies. Since the thalidomide tragedy and the rise of safety–obsessed consumerism, we view clinical research as a necessary evil and something to fear. An over-emphasis on safety permeates all aspects of the Barrier System."

Dr. DeFelice continued, "Often, the media labels clinical research as 'human experimentation.' This connotes an evil act. If an astronaut dies, he's considered a hero. If, however, a patient in a gene study dies, all hell breaks loose. The doctor and hospital are somehow considered as baddies. The FDA and IRBs, responding to pressure, create further regulations and rules that profoundly inhibit clinical research and medical discovery which, ironically, are welcomed in the name of safety. What is ignored is the primary concern of patients – to be cured!"

I asked Dr. DeFelice to give us a simple example what best demonstrates our cultural blind spot to the critical importance of clinical research. Without hesitation, he replied, "Rock Hudson," the famous movie star who died of AIDS in the early phases of the epidemic. "He was a man who was well–liked and well–known to most Americans. Inaccurate media coverage had produced a pervasive national fear of an AIDS epidemic. There were no effective therapies back then.

"An anti–viral drug was in the research phase in France which might have helped Rock Hudson. But the FDA ruled that it didn't meet their requirements and could not be given to Mr. Hudson in the United States. He had to fly to France to be treated! He should have been able to be treated with this drug in the United States."

The popular TV show, *Good Morning America,* learned about Dr. DeFelice's position and invited him and the head of the FDA to a debate. "I sincerely believed that this was the golden opportunity to finally pierce our cultural blind spot about clinical research," Dr. DeFelice said. "I stressed that Mr. Hudson should, for example, be able to receive the therapy at Memorial Sloan-Kettering where the experts are. The FDA policy on clinical research is a huge barrier and should have no role in this early medical discovery phase."

> "I've asked hundreds of men and women in different walks of life, 'When was the last cure?' The overwhelming response has been silence, coupled with blank faces"

The FDA official was evasive, not addressing Dr. DeFelice's point. "I was confident I made the point clearly," he said, "and fully expected that I had started a national discussion on the urgent need to reduce the barriers to early clinical research. *Good Morning America* has millions of viewers and the AIDS phenomenon was of great national concern bordering on near hysteria as if it were another bubonic plague. It seemed to me to be a perfect media storm.

"I alerted Patricia Park, my indispensable sidekick for over 40 years, to 'man' the foundation telephone. The response? Zero! And I mean zero! Not one call from the media, the foundations, the medical community or individuals. If that's not a cultural blind spot, what is? And who pays the price? The defenseless patient!"

Rock Hudson's diagnosis with AIDS was a huge story. The thousands of others who were ill and dying was a big story. What, then, could account for the lack of attention to the need for clinical trials?

"Joe, I wish I knew, but I have a theory. Our society is simply not interested in the general issue of why we don't have cures. Over the years, I've asked hundreds of men and women in different walks of life, many with serious and fatal diseases, 'When was the last cure?' The overwhelming response has been silence, coupled with blank faces. The few who did respond mostly mentioned the polio vaccine which happened in the fifties!

"When I informed them that, despite our booming technology, there are few cures, the almost unanimous lack of curiosity and concern regarding the reasons why was and remains striking. When I explain the role of clinical research in medical discovery, blank faces and lack of curiosity still prevailed. Many, influenced by persistent media coverage, mentioned concerns about the dangers of clinical studies. Many more inquired whether there are new therapies on the horizon for what specifically ails them or their family and friends. These experiences bespeak of a blind cultural mindset which is unbudgeable.

"Even Christopher Reeve, the then extremely popular actor who played the role of Superman, couldn't make a dent regarding the importance of clinical research. In the mid-nineties he fell off his horse, partially severed his spinal cord in his neck and became a quadriplegic – paralyzed from the neck down. He later formed the Christopher and Dana Reeve Foundation which, to this day, is dedicated to funding research for cures for spinal cord injury.

"He observed that, although there was much promising research in laboratory studies, particularly with rodents, few were being tested in clinical studies. His emotionally moving declaration, 'If I were only a rat', which basic on-target message is the difficulty of conducting clinical research went virtually unnoticed and unheeded." •

Part II of this article will appear in EP's August 2016 issue, as well as on www.eparent.com

ABOUT THE AUTHOR:

Stephen L. DeFelice, M.D., is the founder and Chairman of FIM, the Foundation for Innovation in Medicine whose mission is to speed up the discovery of breakthrough medical therapies, including cures. He has proposed the Doctornaut Act as the way to discover such cures as well as substantially reduce health care costs. Visit www.fimdefelice.org. He brought carnitine into the United States and guided it through our entire system to obtain FDA approval which now saves the lives of children with the previously fatal disease, Primary Carnitine Deficiency.

PART II *of a* SERIES

PROMINENT PHYSICIAN PROPOSES A CAN-DO WAY TO REDUCE HEALTH CARE COSTS – *BY FINDING CURES*

INTERVIEW OF STEPHEN L. DEFELICE, M.D., BY JOSEPH M. VALENZANO JR.

In Dr. DeFelice's journey with carnitine, he faced every barrier in our medical discovery system. He believes the Doctornaut Act is the only practical remedy and route to achievable solutions.

"My experience with carnitine and our Barrier System would require a thick book that no one would read," he said. "A single tragic story concerning cancer clearly demonstrates this. At WRAIR, the Walter Reed Army Institute of Research, Major James Vick, an energetic cardiovascular pharmacologist and good friend, and I showed in animal studies that carnitine blocked the heart damage caused by doxorubicin. This highly effective, broad spectrum anticancer drug is limited in use because of its cardiotoxicity. Our findings, which have been confirmed by other researchers, raise the possibility that we could increase its dose, kill more cancer cells, and save or prolong lives.

"We, much to our surprise, then discovered that carnitine increases the kill capacity of doxorubicin ten-fold against rodent ovarian cells in culture. Later, a distinguished scientist colleague, as a personal favor to me, showed that carnitine, by itself, dramatically killed human ovarian cancer cells in culture and also added to doxorubicin's kill capacity. Carnitine alone also kills human colon cancer cells in culture as well as some animal types which add to its promise.

"Boy, was I excited! Both carnitine and doxorubicin can destroy ovarian tumor cancer cells. It's also possible to raise the dose of doxorubicin by protecting the heart and kill even more of them. Carnitine, already in hospital pharmacies immediately available to patients, made it possible to administer this combination on the same day it's ordered by the oncologist.

"My friend, Dr. Cavazza, agreed to fund a clinical study that I proposed in late stage ovarian cancer patients with a certain rendezvous with death. But I needed some type proprietary or exclusivity protection which the Orphan Drug Act provides. I was successful in obtaining such status with carnitine in the past and was sure it would be a slam-dunk. But the head of this division, all by himself with no objections, changed the rules, making it more difficult and costly to obtain Orphan Drug status and rejected my application. I'm sure other medical innovators, knowing this, did not even apply. Dr. Cavazza had no choice and reluctantly withdrew his support.

"So I approached a large pharmaceutical company that would have unquestionably benefitted if this low-cost study were positive. Incredible as it may seem, they refused.

"Next, I contacted my colleague and renowned oncologist, Emil Frei, the distinguished Director of the Dana Farber Institute. He was sufficiently impressed with the carnitine-doxorubicin data to propose conducting a clinical study in patients with soft tissue sarcoma. But, for personal reasons, it never happened. He did, however, recommend two famous oncologists to contact regarding the ovarian cancer study, which I did.

PHOTO PROVIDED BY STEPHEN L. DEFELICE, MD FROM THE NEW YORK TIMES

DOCTOR'S UNITE: Dr. DeFelice with Doctor and former Senator Bill Frist. "Before and during the presidential health care debate, we will present the 'Cure Care versus Health Care' initiative. Through our educational and public relations efforts, we will reach influential leaders who will encourage others to join us. Senator Bill Frist's previous support of The Doctornaut Act will be very helpful to us."

"They, and other oncologists I met, all wanted more costly preclinical studies performed before making a decision. I had some good luck and arranged to have the study approved at a local hospital. But patients were tough to come by. I asked a prestigious national foundation that deals with ovarian cancer to help us locate patient volunteers, but they were not interested.

"When I tell this story to people they are incredulous! They simply don't understand how this could happen. My long experience with carnitine and ovarian cancer is difficult to accept, let alone understand.

"After this experience, the gods on Mount Olympus sent me a message that it was time to give up the ship. And so I did. I am not saying that carnitine is a miracle cure because it's not. It's a long shot. But it was the only shot!

"And there's a reasonable theory as to why it might work. Many tumors prefer sugar to feed on. What carnitine does is to make cells eat fat and this effect may actually cause tumors to starve to death or become more sensitive to anti-cancer drugs and the human immune system.

> I asked "Why do you still believe that our next president or even Congress would become advocates of the Doctornaut Act?" Without hesitation, he shot back, "The national debt and the impact of health care costs."

"This is nothing new. In 1931, Otto Warburg won the Nobel Prize for his work on the anaerobic metabolism of cancer cells and their need for sugar. There appears to be a 'Warburg Revival' underway now and this might hopefully be helpful to patients.

"To repeat, we're dealing with an anti-patient cultural mindset. The ovarian cancer patients were at the end of the therapeutic line and doomed to die. And, as I said before, there's carnitine and doxorubicin sitting on hospital pharmacy shelves immediately ready to be administered. What most disturbs me is that patients were not told about the option. It's all part of our invisible Barrier System."

Dr. DeFelice summed up this situation. "What's the general message of this specific experience? The FDA bureaucrats, the corporate physicians, the medical foundations, and the oncologists form an intertwined, complex system that creates obstacles to promising clinical trials. Money reigns supreme. Lots of it would have overcome the barriers to the ovarian cancer study."

Dr. DeFelice paused, looked me straight in the eye, which

PHOTO PROVIDED BY STEPHEN L. DEFELICE, MD

meant something big was coming. "Joe, my experience with carnitine and cancer exemplifies the general nature of our Barrier System. The barriers are the same for all promising therapies. I have had similar experiences with nerve growth factor in multiple sclerosis and a cervical cancer vaccine, to name just two.

"The ovarian cancer story; the example of Rock Hudson on *Good Morning America;* and others examples send an unequivocal message. We have a huge cultural blind spot to even thinking about of having a *Cure Care* policy and an absolute blind spot regarding the essential role of clinical research in medical discovery.

"To repeat, the good news is that the simple, uncomplicated Doctornaut Act is the solution. If, for example, female doctornauts with ovarian cancer had existed in the late 70's, then many patients would still be with us. And this discovery would have led to clinical studies with the combination in other types of cancer.

After Dr. DeFelice described parts of our labyrinthine system, I told him I couldn't see how The Doctornaut Act could change it—and he surprisingly agreed!

"The system cannot be changed," he asserted. "It is embedded in our culture, so you have to go add to it. The Doctornaut Act is simply an uncomplicated addition. But here's the other good news. If the carnitine-doxorubicin combination destroyed ovarian cancer tumors then, by public demand, the pressure would be so great that the administrative system would have to make it available to doctors and patients as soon as feasible. And don't forget, that doctors are not bound by the FDA to treat patients for non-approved uses. Public pressure will play a huge role in all major medical breakthroughs.

I then challenged him, "You have tried unsuccessfully for over 40 years, what makes you believe that now is the time to seize the moment?"

Dr. DeFelice replied, "Our culture is rapidly changing its habits and values. People, particularly baby boomers, are paying more attention and are better informed. Although there is much misinformation from the media regarding health and medical issues, the public does hear about promising medical advances. This may help create a sense of urgency which we sorely need to bring about change. Also, there's also the cost of medical care which combined with the sense of urgency can change our cultural mindset."

DeFelice switched gears again and said, "Speaking of the media, notice that, after reporting on a potential new therapy, they routinely report that it will take a long time before it reaches the patient. They never—and I mean never—explain why! They themselves haven't the slightest understanding of the Barrier System and it is tough to find experts to ask why this is so."

> "Did you know that future health care cost projections do not include the discovery of cures? This presidential debate has aroused the interest of the public and media as never before. The people are now listening."

I asked Dr DeFelice the bottom line question. "Would physicians be willing to be Doctornauts?" It's interesting to note that in Michael Mannion's book, *A Maverick's Odyssey,* about Dr. DeFelice's quest to conquer disease, a few of his physician friends who are sympathetic to his mission were not convinced doctors would volunteer. Dr. DeFelice dismisses their beliefs for a variety of reasons. Specifically, he learned in his work with prisoner volunteers for clinical trials how strongly people are altruistic and want to help others.

In his research unit in a state prison, and at WRAIR, where he collaborated with two other prison facilities, he serendipitously discovered carnitine's role in cardiac disease in one of his prisoner volunteers. *This opened the doors to its development for Carnitine Deficiency in children.*

Dr. DeFelice suddenly smiled. This time it was a cynical one. "Would you believe that later on, the FDA virtually closed down prison research facilities? This created another significant barrier to discovery. And it robbed prisoners of the right to be noble and courageous. The barriers never stop. Once more, who pays the price? The patient!"

In 1983, because of his personal interest in the promise of natural substances, the Foundation for Innovation in Medicine conducted a physician survey asking, "Would you, as a physician-patient, want the privilege to volunteer for clinical research of natural substances under the supervision of a physician-clinical researcher without any FDA, institutional or other restraints?" Over 50 percent said they would. Women physicians were as bullish as the men.

Today, there are over 900,000 U.S. physicians in the U.S. If only 10 percent volunteered, there would be 90,000 Doctornauts, a substantial number for early discovery phase studies where generally only small numbers of patients are evaluated. Dr. DeFelice suggested that foreign physicians might also be permitted to be doctornauts in the United States. Why not?

"Dr. DeFelice, I understand your general concept but how, specifically, would Doctornauts speed up medical discovery?"

"Joe, generally speaking, Doctornauts would participate in small, short-term clinical studies with potential therapies that offer more than ordinary promise," he answered. "Doctornauts are not suited for long term clinical studies, such as whether a cholesterol-lowering agent prevents heart attacks. Large numbers of non-patented, logical combinations of promising therapies, as well as natural substance therapies, will be tested. This will not happen without the Doctornaut Act. Doctornauts are major door openers which will, without doubt, expand our base of medical innovators.

"Here is another great example," he continued. "Genetic therapy, particularly the newly discovered CRISPR gene-editing technology, is controversial. People understandably fear it will alter human

nature in ways unknown. Costly and time-consuming barriers will certainly be erected before the first dose is given in any clinical study, let alone subsequent ones. This is bad news for orphan or rare diseases and disabilities. There are about 7000 of them; 80 percent are due to genetic abnormalities.

"It's estimated there are 30 million orphan disease patients in the United States, many of them who are children. But with Doctornauts, the barriers would be reduced and discoveries made that could lead to new treatments for children. If, for example, a drug is effective in doctornauts with leukemia, it could also be given to children. It's a best kept secret that the vast majority of drugs cannot get to the brain because of the blood-brain barrier. A recent really exciting study in mice reported that, using viruses as the carrier, not only drugs, but also genes can enter the brain. If studies in doctornauts prove this to be true, then this method can be employed in children with multiple types of neurological disabilities and disease and would lead to dramatic medical breakthroughs.

I asked "Why do you still believe that our next president or even Congress would become advocates of the Doctornaut Act?" Without hesitation, he shot back, "The national debt and the impact of health care costs."

I asked him to elaborate. "Over the years," he began, "I've come to know conservatives and liberals both in the House and the Senate, as well as influential elites who impact public opinion and public policy. About 25 years ago, I met with one of the most liberal members in the House of Representatives, a thoughtful and sincere man who is still there. I explained the rationale behind the Doctornaut Act, seeking his advice on how to move the Congress to enact it.

"After a long moment of silent reflection, he confidently answered, 'Make it clear how your doctornauts will reduce health care costs. That will get our attention because no one knows how to substantially reduce costs except by political suicide.' He was, of course, referring to making big cuts in Medicare and Medicaid services which even President Reagan, in his cost reduction initiative, left untouched.

"I told him that the cost reduction argument may not convince opponents who would raise a legitimate argument: breakthrough therapies would be expensive and increase costs. He agreed that this could be a problem and asked if I saw a solution.

"I smiled and answered, 'Capitalism.' He also smiled for he's not a great fan of it. I explained that, in our dynamic market system, both expensive and inexpensive therapies would soon be discovered and compete with each other in the medical marketplace.

Exceptional Parent will join forces with Dr. DeFelice. We plan to form a group of dedicated moms with children with disabilities and diseases, Mothers for Doctornauts, who are committed to spreading the message.

"For example, the estimated cost for Alzheimer's by the year 2050 is $20 trillion—greater than our current national debt. Also, the money saved by curing Alzheimer's could be used for research on diabetes, autism and other diseases. It's a win-win situation.

"Did you know that future health care cost projections do not include the discovery of cures? This is mind-boggling and confirms our cultural blind spot that they won't happen.

"As I said before, this presidential debate has aroused the interest of the public and media as never before. The people are now listening. Before and during the presidential health care debate, we will present the *Cure Care versus Health Care* initiative. Through our educational and public relations efforts, we will reach influential leaders who will encourage others to join us. Senator Bill Frist's previous support of The Doctornaut Act will be very helpful to us.

"What will also help is the Act's simplicity. Unlike the 2000-page, labyrinthine Affordable Care Act, ours could be about 12 pages long and can be read and understood within an hour!"

Finally, I was curious to learn about his marketing strategy. "I'm depending on what I call a 'Pascal moment.' The brilliant French thinker observed that small things can have big impacts. For example, if Cleopatra had a really big nose, Julius Caesar would not have fallen for her. Roman history—and the history of Western civilization—would have been different. Our Pascal moment will be a small, but focused, public education effort that would hopefully have a large impact. Much depends on timing, luck and prayers. And there's no doubt that I'll be asking God for any help he can give me. It's now or never for the Doctornaut Act. Let's give it our best. We need dedicated leaders to join us. I can't do it alone."

Well, I told him that *Exceptional Parent* certainly will join forces. We plan to form a group of dedicated moms with children with disabilities and diseases, Mothers for Doctornauts, who are committed to spreading the message.

In the final analysis, Dr. DeFelice is the one person who can coordinate and implement the entire approach. Let's hope that he convinces our next president to seize the moment and successfully push for the enactment of The Doctornaut Act. •

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ABOUT THE AUTHOR:

Stephen L. DeFelice, M.D., is the founder and Chairman of FIM, the Foundation for Innovation in Medicine whose mission is to speed up the discovery of breakthrough medical therapies, including cures. He has proposed the Doctornaut Act as the way to discover such cures as well as substantially reduce health care costs. Visit www.fimdefelice.org. He brought carnitine into the United States and guided it through our entire system to obtain FDA approval which now saves the lives of children with the previously fatal disease, Primary Carnitine Deficiency.