“Breakthrough” treatments that we don’t really need (part three)

Back by popular demand: the third installment in my series *“Breakthrough” treatments that we don’t really need*. I tried to contain myself to just two newsletter articles, Part 1 and Part 2, but there were too many instances to relegate to the cutting room floor. Seems not a week goes by that we’re not regaled with news of a “breakthrough” treatment that promises relief from one or another malady. Most of these are expensive, and some come with an array of harrowing side effects. Plus, they’re often of marginal efficacy. Isn’t it time that we hit pause on our headlong rush to “progress” and call instead for refocusing on safe, reliable, economical solutions predicated on root causes?

Anti-Smoking Vaccine: No question that smoking sickens and kills millions worldwide. And despite a raft of therapies, both behavioral and pharmaceutical, success is elusive and many smokers relapse. Even a significant proportion of COPD patients (~40%), who daily face the direct consequences of their habit, continue smoking despite knowing that they have a suffocating disease.

Enter the “nicotine vaccine”. According to a review:

“The nicotine vaccine is intended to treat drug abuse through active immunisation by inducing nicotine-specific monoclonal antibodies (nic-mAbs) to sequester and reduce

nicotine distribution inside the brain."

What could possibly go wrong?

First of all, the vaccines are of dubious efficacy. A Cochrane review of four studies comprising 2642 smokers concluded:

"None of the four included studies detected a statistically significant difference in long-term cessation between participants receiving vaccine and those receiving placebo . . . The evidence available suggests nicotine vaccines do not induce compensatory smoking or affect withdrawal symptoms . . .There is currently no evidence that nicotine vaccines enhance long-term smoking cessation."

A paltry quit rate of 11% was seen in *both* the vaccine recipients as well as the unvaccinated controls.

Moreover, there are serious questions as to the desirability of abrogating the brain's response to nicotine, which is the mode of action of these vaccines. Studies have shown, for example, that nicotine improves memory and attention, lowers the perception of pain and physical stress, and may even be a bulwark against Parkinson's Disease and dementias.

Nicotinic receptors operate even in the brains of non-smokers; they work via the action of vitamin B3, metabolites of which, like nicotinamide adenine dinucleotide (NAD+)—a coenzyme for redox reactions, making it central to energy metabolism—are molecular cousins of nicotine.

For now, anti-smoking vaccine trials seem to have ground to a halt, despite rosy predictions of huge profitability.

Because nicotine, while addictive, may have long-term salutary health effects when not teamed with tar and chemical additives, I've encouraged smokers to kick the butts and instead resign themselves to long-term and even lifelong nicotine substitution via gum, lozenges, or patches.

Cures for Muscular Dystrophy: MD is actually a collection of genetic diseases that cause progressive muscle weakness. Duchenne Muscular Dystrophy is the most common form, affecting male children from around the age of four, comprising around half of all cases of MD. Various medications have been employed with sketchy impact on progression; there is currently no cure for MD. The disease affects an estimated 10,000 to 12,000 children in the U.S.

As with other genetic diseases like sickle cell, for which the first genetic therapy was recently authorized, therapies like CRISPR may eventually offer promise of a definitive fix. Surely, it's heart-rending for parents to watch helplessly while a child afflicted with MD becomes inexorably debilitated without prospect for relief.

Nonetheless, the advent of gene therapies for MD has stirred controversy. New treatments are predicated on introducing a gene that manufactures a copycat of a protein called "dystrophin" via a series of infusions. Dystrophin is conspicuously missing from the muscle tissues of boys with Duchenne Muscular Dystrophy.

Sounds plausible, but results are equivocal. Moreover, the cost of a course of treatment may be as high as three million dollars. Under an FDA program that enables "accelerated approval" for sufferers of serious diseases without recourse to any other therapies, gene treatments for MD qualify for approval absent the stricter

clinical trial criteria applicable to mainstream drugs. Even a scintilla of evidence of an effect, as with cancer and Alzheimer's drugs, allows them to be marketed. Medscape reports:

"Three genetically targeted drugs for Duchenne muscular dystrophy (DMD) — eteplirsen, golodirsen, and casimersen — cost the US health care system more than $3 billion between 2016 and 2022, despite a lack of confirmatory efficacy data, a new analysis showed."

Unlike other neurodegenerative conditions like Alzheimer's, there's no indication that MD is amenable to lifestyle modification. But there's intriguing evidence that creatine monohydrate supplementation—inexpensive and readily available over the counter—may slow its progression in children. Even some pediatric hospitals recommend it.

There are concerns that the hopes of parents of children with MD may be dashed, and taxpayers left on the hook for the steep costs of iffy treatments, because accelerated approval exempts developers from stringent post-marketing studies that might eventually show their drugs are ineffective. There are risks that unscrupulous Pharma entrepreneurs could game the system.

ALS Game-Changer: A case in point is Amylyx Pharmaceuticals' voluntary withdrawal of their drug Relyvrio from the ALS marketplace. There are currently at least 40 drug candidates to address the elusive condition; Relyvrio obtained accelerated approval in 2022, but subsequent analysis showed the drug didn't work to slow ALS progression. Amylyx generously offers that ALS patients who believe they were helped by Relyvrio will now be eligible to receive the drug for free—but how plausible is it that they're not merely experiencing a transient placebo benefit?

Meanwhile, the recent PARADIGM trial suggests that a combo of two traditional drugs—Cipro and Celebrex—can meaningfully slow the progression of ALS.

Free Lunches for Rich Kids: And while we're at it, though it's not a drug fix, here's an example of a wasteful, albeit well-intentioned, expenditure of tax dollars with dubious benefits.

The Wall Street Journal reports:

"In September 2023, the USDA's Food and Nutrition Service finalized a rule that expands the number of students who qualify for reduced lunches during the school year. If a mere 25% of a public school's students meet the requirements, 100% of its students will be eligible to receive the benefit. The rule imposes no income limits, meaning middle- and upper-class children will get subsidized meals."

I don't know about your kids, but few youngsters can avoid the temptation of swapping out a considerately packed lunchbox apple for a generous helping of Oreos or Little Debbie's Cakes, provided by a classmate, or even conveniently available in vending machines that share revenues with school districts. Worse yet, they can be lured to a nearby strategically located Taco Bell or MacDonald's, which were already a burgeoning trend even in the antediluvian era when I went to high school.

Congress and the administration are selling these programs on the worn premise that "no child should go hungry", but the evidence is that most kids these days are over-nourished with ultra-processed foods, leading to unprecedented rates of childhood obesity and diabetes.

Waste is a problem with these school lunch programs; a recent study shows the U.S.

leads the world in discarded food. Researcher Christine Costello notes: *"A study in Sweden showed that plate waste accounted for 23% of total food served; in Italy it was between 20% and 29%; and in Spain about 30% . . . In our study, we found plate waste ranging from 27% to 53% of the food served."*

No wonder. An investigation by the New York Post of the "new and improved" school lunch program in New York City—one of the most progressive in the country—revealed:

"New York City kids would rather starve than eat the cut-rate cafeteria food being rolled out across the city – with options such as chickpea stew, veggie burgers and zucchini-carrot breakfast bread filling up public school trash cans."

Meanwhile, Consumer Reports has called for the USDA to remove Lunchables from schools' lunch menus.

Extra Cheesy Pizza anyone?

This concludes our series *"Breakthrough" treatments that we don't really need*, but I'd be happy to entertain your suggestions for additional contenders—we might include your offering in a future bonus article. Send ideas to questions@drhoffman.net.