# Orphan Drugs CP

## 1NC

### 1NC shell – pharma

#### CP text: The member nations of the WTO should [insert plan mandate] except for orphan drugs.

#### IP protections for orphan drugs are key to the pharma industry – it’s key to their market strategy

Houldsworth 19 (Adam Houldsworth Author | Life sciences reporter, 29 Oct 2019, “Four key IP implications from the rise of orphan drugs” <https://www.iam-media.com/law-policy/four-key-ip-implications-rise-orphan-drugs>) [Twinz]

A recent report from Clarivate Analytics provides the latest reminder of the growing importance of orphan drug innovation to the life sciences industries. The 2019 Centre for Medicines Research International Pharmaceutical R&D Factbook points out that of the 57 new molecular entities approved by the US Food and Drug Administration in 2018, 22 had an orphan drug designation, signifying that they target a rare disease. Between 2010 and 2018, it notes, the number of addressable patients for each drug approval fell by 15%. This echoes other data that suggests an increasing significance for drugs aimed at treating rare diseases. According to EvaluatePharma’s 2019 Orphan Drug Report, worldwide sales from orphan drugs are forecast to grow by 12.3% CAGR between 2019 and 2024. This is double the rate for non-orphan drugs. Medicines approved for rare diseases will provide 20.3% of global prescription revenues by the end of this period. During this period, they are set to provide a third of the industry’s R&D pipeline sales. Requests for orphan drug designations have risen precipitously over the past decade. In 2009 – itself a record-breaking year – there were 250 applications to the US FDA, compared to 346 in 2013, 467 in 2014, 472 in 2015, 582 in 2016 and just over 500 in both 2017 and 2018. The same period has seen a large increase in the number of designations awarded in the US. No year before 2011 saw more than 200 designations, but every year since 2013 has witnessed more than 250, with over 300 in each year since 2015 - and a record 477 awarded in 2017. What’s more, the number of orphan products (including repurposed products) approved each year in the US has also increased dramatically. It reached record numbers in 2017, when 81 products were approved; and again in 2018, which saw 91 drugs given the green light (there had never been more than 30 orphan drug products approved in a year until 2013). This trend has potentially important implications for intellectual property. 1. Growing importance of regulatory exclusivities for IP professionals One clear consequence for IP strategies is that the regulatory market exclusivities offered by drug administrations in several key jurisdictions will increase in significance. Since the 1983 Orphan Drug Act, the US has awarded seven years’ exclusivity after market approval – as well as tax credits – to innovators of orphan-designated drugs. This applies to drugs that target conditions with fewer than 200,000 sufferers in the country. During these seven years, the FDA will only grant marketing approval to another version of the same drug for the same indication if that newer version can be shown to be to be “clinically superior”. Depending on the term of a product’s patent protection, this exclusivity can significantly extend a drug’s monopoly. The EU adopted similar provisions in 2000, since then it has granted 10 years’ regulatory exclusivity to orphan drugs; while an extra two years’ protection is awarded if the drug is a paediatric product. The European Medicines Agency awards orphan designations to products targeting serious conditions suffered by no more five in 10,000 Europeans, provided that the drug would otherwise produce insufficient returns to justify development costs and no other satisfactory product exists. In Europe, this monopoly excludes all “similar medicinal products” for the same indication, not just versions of the same product. But orphan drug exclusivity can be cut short to six years if by the fifth year the drug has reached a level of profitability that means it no longer qualifies for orphan drug status. And, as in the US, other similar products demonstrating clinical superiority may be approved during the exclusivity period. Similar provisions exist in Japan and Australia. As the orphan drugs gain in commercial importance for the industry and specific companies, gaining and maintaining these exclusivities will become an even more central part of overall IP strategies. The distinctive case law around orphan drug exclusivities will be more important to pharma patent professionals, as will knowledge of relevant regulatory processes and standards. 2. Political controversies over pharma IP to be exacerbated Another potential consequence of the rise of orphan drugs may be a growth in public concern about drug prices, leading to a heightening of existing political tensions around life sciences patents. As detailed previously by IAM, pharma IP (and patent owner strategies) have been severely criticised in recent years. This is especially so in the United States, where they have been blamed for underpinning high drug prices. As a consequence, we have seen several legislative proposals seeking to rein in pharma IP rights. Orphan drugs risk fuelling these controversies because their mean cost per patient is significantly higher than the mean cost of other drugs. According to EvaluatePharma, the top 100 US orphan drugs cost on average 4.5 times as much per patient as non-orphan drugs, with a mean cost of $150,854 per patient in 2018, compared to $33,654 for other treatments. In general, this is because rights holders are seeking to recover costs and make a profit from what are, by definition, smaller patient populations. But, given the seriousness of the conditions targeted by orphan drugs, and the fact that there are no alternative treatments for many rare diseases, such prices risk calling into doubt public confidence in pharma IP rights. In the US, orphan drug exclusivities themselves have been subjected to serious scrutiny in recent years, with Senator Chuck Grassley announcing an investigation into potential misuses of the orphan drug programme in 2017. In 2018 and 2019, a plethora of news articles (such as this one) have called for reform. And in the UK, the National Health Service’s long-running dispute with Vertex Pharmaceuticals over Orkambi – resolved just last week – led to calls for the use of compulsory licensing. 3. More IP deals and collaborations The growing importance of orphan drugs is likely to accentuate a broader trend towards the acquisition and licensing of products invented by small and medium-sized biotechs by big pharma. Against a background of rising R&D costs, and pressures to replenish pipelines to mitigate the second patent cliff, big pharma has in recent years sought to buy or license-in promising innovations from other companies, usually smaller entities. This trend is especially pronounced in the orphan drug space, where inventions are produced overwhelmingly by small and medium-sized biotechs. Nimbler innovators have traditionally dominated this space because of its requirement for specialised knowledge and laser focus on particular indications. Indeed, the US National Organization for Rare Disorders – which lobbied for the 1983 Orphan Drug Act – claims that the American biotech industry is a “child” of that legislation. If one looks at the EvaluatePharma’s top 20 orphan-designated R&D drugs, all those in the hands of big pharma companies have been either acquired – as with Celgene’s Liso-cel and Novartis’ Zolgensma – or licensed-in (like Roche’s Risdiplam and Sanofi’s Isatuximab) from other companies. The only organically developed products in this list are in the hands of medium sized companies, such as Blueprint Medicines, bluebird bio and Deciphera Pharmaceuticals. And even many of these are being developed in collaboration with larger entities. For example, Galapagos’ GLPG1690, to which Gilead has exclusive licensing options; and bluebird’s bb2121, which is partnered with Celgene. What’s more, it is striking to note that of Roche’s abundant portfolio of orphan-designated drugs – Herceptin, Perieta, Tecentriq, Avastin, Rituxan, Gazyva, Venclexta, Kadcyla and Alensa – none were originally developed in-house. 4. More emphasis on personalised drugs While many orphan drugs are not personalised treatments, they account for a significant chunk of the medicines in this category; and the rise of rare disease treatments is happening hand-in-hand with the growth of personalised and precision medicines – which are also expected to increase their revenue by over 11% CAGR between 2017 and 2024. As such, the increasing significance of understanding the distinctive challenges of, and divergent international rules for, patenting personalised medicines will be an important by-product of the global increase in orphan drugs. This is one of the reasons why issue 98 of IAM magazine will include in-depth guidance on how best to protect personalised medicine innovations from Frances Salisbury of Mewburn Ellis.

#### Especially true in the context of the plan – orphan drugs are the only way to ensure pharma stays stable – that’s key to innovation

Sharma et al. 10 (Aarti, Massachusetts College of Pharmacy and Health Sciences member of Department of Pharmaceutical Sciences and specializes in Medicinal Chemistry, Pharmacology, Organic Chemistry., Abraham Jacob, Manas Tandon, and Dushyant Kumar., December 2010., “Orphan drug: Development trends and strategies”, <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC2996062/> [twinz])

The growth of pharma industries has slowed in recent years because of various reasons such as patent expiries, generic competition, drying pipelines, and increasingly stringent regulatory guidelines. Many blockbuster drugs will loose their exclusivity in next 5 years. Therefore, the current economic situation plus the huge generic competition shifted the focus of pharmaceutical companies from the essential medicines to the new business model — niche busters, also called orphan drugs. Orphan drugs may help pharma companies to reduce the impact of revenue loss caused by patent expiries of blockbuster drugs. The new business model of orphan drugs could offer an integrated healthcare solution that enables pharma companies to develop newer areas of therapeutics, diagnosis, treatment, monitoring, and patient support. Incentives for drug development provided by governments, as well as support from the FDA and EU Commission in special protocols, are a further boost for the companies developing orphan drugs. Although there may still be challenges ahead for the pharmaceutical industry, orphan drugs seem to offer the key to recovery and stability within the market. In our study, we have compared the policies and orphan drug incentives worldwide alongwith the challenges faced by the pharmaceutical companies. Recent developments are seen in orphan drug approval, the various drugs in orphan drug pipeline, and the future prospectives for orphan drugs and diseases.

#### Pharma innovation is the only line of defense from super bugs causing extinction

Charlton 20 (Emma Charlton, Senior Writer, 20 Nov 2020, “The looming health catastrophe that could be more deadly than COVID-19” <https://www.weforum.org/agenda/2020/11/superbugs-health-risk-antimicrobial-resistance/>) [Twinz]

Superbugs. You’ve probably heard of them, but did you know they’re one of the biggest threats to global public health? Left unchecked, these drug-resistant bugs could kill millions of people every year with the damage to health potentially dwarfing that of COVID-19, according to the AMR action fund. That makes antimicrobial resistance, or AMR, a top 10 global public health threat, according to the World Health Organization, which is raising awareness and promoting ways forward with World Antimicrobial Awareness Week. Have you read? This simple reaction could make antibiotics more effective against drug-resistant bacteria COVID-19 is putting millions of people at risk of blindness Risks arise when bacteria, viruses, fungi and parasites change over time and don’t respond to the drugs that have been developed to keep them in check. Strong resistance Resistance is increasing, partly because antimicrobials have been overused since their discovery, and partly because poor sanitation and hygiene allow resistant strains to spread. In farming, antibiotics are often given to animals to boost their growth or to prevent diseases from spreading when livestock are kept in cramped conditions. COVID-19 has added another layer, with antibiotics being prescribed to people around the world, even though it is caused by a virus, not by a bacteria, the WHO says. AMR Action Fund bacteria viruses fungi drug antimicrobial resistance global public health threat Deaths could increase rapidly if no action is taken. Image: AMR Action Fund Tackling resistance matters because the problem has the potential to spiral, with the AMR Action Fund estimating that deaths from antibiotic-resistant infections could rise to around 10 million a year by 2050, up from around 700,000 in 2019. And it could cost the global economy as much as $100 trillion between now and 2050, according to the International Federation of Pharmaceutical Manufacturers & Associations, IFPMA. “The coronavirus has really driven home how vulnerable we are as a society to contagious diseases,” says Lars Rebien Sørensen, chairman of the Novo Nordisk Foundation, which helps fund the AMR Action Fund. “2,000 people die every day due to antimicrobial-resistant infections. Even if we start doing everything we can today, this number will increase before it will drop. If we fail to act, a catastrophe is looming.” The WHO global action plan seeks to improve awareness of the issue, bolster research, improve sanitization, cut back excessive use of antimicrobial medicines in human and animal health and invest in new medicines to act against the superbugs. What is the World Economic Forum doing about epidemics? Show Prevention focus At the moment, the bacteria are winning the race, morphing faster than drugs are being developed to counter them, the AMR Action Fund says. And that’s partly because of the poor business case: development costs cannot be covered through sales. While pharmaceutical companies are racing to find a vaccine for COVID-19, research and development of new antibiotics has slowed, according to Pew research. Now the WHO is calling for a bold, unified agenda focused on prevention and finding new medicines. AMR Action Fund bacteria viruses fungi drug antimicrobial resistance global public health threat The WHO is calling for action to fill the gap. Image: Pew Charitable Trusts Research Seeking to redress this, the AMR Action Fund has raised $1 billion from major pharmaceutical companies to invest in biotech and plans to bring as many as four new antibiotics to patients by 2030. “There is currently no viable market for the development of new antibiotics,” says Kasim Kutay, CEO of Novo Holdings, which administers the investment in the AMR Action Fund on behalf of the Novo Nordisk Foundation. “As a result, antibiotics that are in the early stages of development never reach patients because of a lack of funding for the later stages of clinical research. The AMR Action Fund is an important part of the solution to this.” Until new antibiotics are found, the US Centers for Disease Control and Prevention advocates good general health practices, like keeping your hands clean, getting vaccinated, only using antibiotics when they’re really needed, and preparing food in a hygienic way. Even so, it’s likely to be a long battle. “AMR is a complex problem that requires a united multisectoral approach,” the WHO says. “Greater innovation and investment is required in operational research, and in research and development of new antimicrobial medicines, vaccines, and diagnostic tools.”