7. Medicoeconomic evaluations of innovation: methods and research tools

Hello. I am Isabelle Durand-Zaleski Professor of Medecine at University Paris-Est Créteil. I work for the public hospitals of Paris as leader of the research team on health economics called URC Eco. We do economic assessments of health technologies. The policy maker considers several factors when faced with a rather large supply of innovations. Those may be medical devices, diagnostic techniques or organizational changes in health care such as telehealth or connected devices as well as skill transfers in advanced nursing practices. Policy makers want to know the extra cost of these innovations and the health benefits their implementation brings. They want to understand how this technology could benefit the population how much it will cost and how much it will save compared to the current situation. To assess health technologies we rely on methods developed in biomedical research. Our goal is to identify the benefits that can be directly attributed to the implementation of this technology as well as spending, both incurred and avoided. The findings from our innovation assessments are then used to explain what extra costs need to be absorbed to obtain the health benefit. How do we assess health technologies? Methods can vary. The one we usually rely on – though it may be different elsewhere – is having our team work alongside clinicians on the same clinical trial. Our research is prospective. We study at the same time the health outcomes and the costs generated by the implementation of a technology. Other teams favor the use of models because models can generate findings about health benefits and costs on the very long term. A clinical trial cannot. Clinical trials can be assessed in a variety of ways. Whenever it is possible, most often when assessing a drug we have double-blind controlled randomized trials which are akin to a gold standard in biomedical research. But very often, when testing devices, diagnostic techniques and organizational changes, especially double-blind randomization, is not possible. Even controlled randomization is rather hard to set up for practical management reasons. Sometimes you cannot have controlled randomization either because there are no control groups available or because your investigators cannot organize individual randomization or because you cannot do direct comparisons, etc. In that case, you can use alternative designs and control groups. When, in a prospective study, you cannot have a group that would not benefit from the intervention, you have to look at what happened in previous groups, which we can usually find in medicoadministrative databases such as hospital databases or France's SNIIRAM database. When simple randomization is not available we can rely on other randomization methods. There is an interesting review in the literature that lists all those alternative study designs. Remember they are harder to implement and less thorough than individual randomization and often require larger sample sizes. Examples include cluster randomized trials and stepped wedge trials, that is to say cluster randomization that accounts for time – very useful when you think the intervention would benefit people but cannot be implemented for everyone at the same time. There is also time series monitoring and, when the impact of the intervention varies widely between your subjects you can try using Zelen or preference designs. Those allow your patients some control over which intervention they want to undergo. On the following graph all of these study designs are represented. There is a specific slide for stepped wedge trials – remember, cluster randomization that accounts for time. Now let's talk about a few examples of studies and some findings we obtained. I want to talk about a diagnosis in a randomized trial about a model-based diagnostic approach and about a preventive drug.

Our first study contrasted two diagnostic approaches to compare the costs and outcomes of a non-invasive strategy – a CT coronary angiogram – versus an invasive coronary catheterization in diagnosing angina pectoris. What makes this study unique is that it shows that the non-invasive CT coronary angiogram generates slightly fewer diagnoses in low-risk patients but lowers costs substantially. Look at the graph on the right showing the final outcome of the cost-effectiveness study. Along the horizontal axis we see the cost difference: the cluster of points represents the confidence interval of the results. It is below zero meaning that the intervention is within the cost-reduction zone, but it is also on the left of the graph which means that the outcomes are slightly below those of the invasive approach. The second study compared several approaches of care monitoring, and screening of liver cancer in patients with cirrhosis. We compared an optimal strategy with medical monitoring and an ultrasound every six months to the approach in use today with much looser patient monitoring. We then looked at what happens when a cancer diagnosis is given and when that diagnostic leads either to radiofrequency treatment to surgical resection or a liver transplant. The clusters of colored points on my left show the relationship between the outcomes and extra costs while accounting for the difference between the French health care system and the American one. On the slides you can see the areas with red dots are always less expensive but as efficient as the areas with blue dots. That just means that some fairly conservative care approaches like radiofrequency or simple surgery resection are less expensive but just as efficient as a more complex options, e.g. liver transplants. My last example is about using pre-exposure prophylaxis at the request of patients at risk of HIV infection. This was the Ipergay trial: a double-blind drug trial with controlled randomization conducted in France and Canada. Gay men at risk of HIV infection were offered a preventive option in the form of a retroviral drug taken before risky sexual encounters. We conducted an economic evaluation of the trial. You can see on our graph after how many years of exposure the on-demand prophylaxis approach remains beneficial to the health care system. The three colors represent three prices of the drug. Red accounts for the brand-name drug price in France. Blue stands for the generic drug price in France and green represents the generic drug price online. The graph shows that for exposure periods lasting anywhere from 8 to 20 years, it is always better for the health care system to offer on-demand prophylaxis, not only because it limits cases of infection, but also because it limits spending associated with these cases: you can see that for 8 years with the brand-name drug 13 years with the French drug and 20 years of the Indian drug sold online this option remains cheaper than treating the infections that result from risky encounters. To conclude, let's look beyond these findings. We always try to consider if our findings contribute to a change in practices, to a change in the way patients are taken care of and how the innovation spreads. And before that, – as per advice from the research section of the Ministry of Health – it is always good to make use of your study findings to determine which areas of research should be top priority and to check for interventions that would not be less efficient or just slightly and acceptably so while being much less expensive than existing interventions. Part of health economics means assessing innovative technologies and creating research priorities for health technologies.

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