Exploring Survey Data on Health Care

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Lecture - 39
Randomized Control Trial (RCT)

Welcome friends, once again to my module of NPTEL on exploring healthcare survey data. We are on the verge of completion since we are dealing with the last week of the session. I hope you must have been enjoying our all-hands-on experiences with healthcare data.

We have already initiated estimation with the use of evaluation techniques. I know that this is the need of the hour, where we are evaluating various programs.

The government and even many NGOs, and many corporate houses are targeting to engage the researchers, who have a repository of knowledge with evaluation techniques. That is how we have thought of giving a module, where you guys have enough understanding of evaluation techniques.

We are not giving detailed information about all those techniques, but we are for sure giving you the capsule requirement that may address important dimensions of evaluation, designs, and techniques. We are here on the last week and 2 lectures we have already taken on evaluation models or evaluation techniques. The first one is an introduction, the second one is a PSM. Here we are discussing randomized control trials and no introduction is required to explain randomized control trials.

However, it is good to mention and refer to the latest Nobel Prize that was given in 2019 to Professor Abhijit Banerjee and their team. You must be surprised to know that nowadays, around 80 percent of the research in developing and non-developing countries are utilizing RCT designs or evaluation techniques. So, since they are using this obviously, the person who is having knowledge on this will be most demanded.

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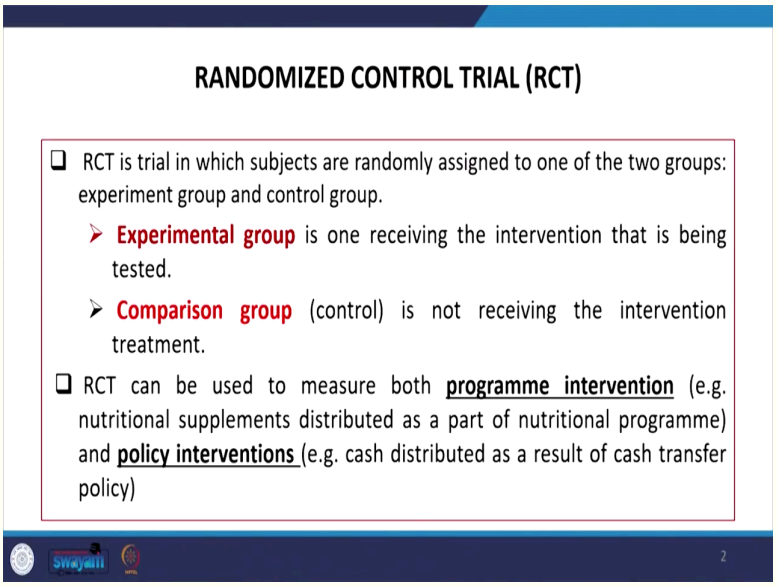

So, let us go further in the understanding of randomized control trial. RCT is a trial in which subjects are randomly assigned to one of the two groups which are called the experimental group and another is called control group. But in PSM we are not dividing it by experimental group or control group.

Though we are saying there are some counterfactuals and treatment groups, here we are specifically emphasizing these two. That is why the PSM was defined as quasi-experimental, but this one is in fact called experimental design. So, what do you mean by experimental group?

The experimental group is the one receiving the intervention that is being tested. What do you mean by comparison group? They are also called the control group which is not receiving the intervention treatment. So, RCT can be used to measure both program interventions that are nutritional supplements distributed as a measure of nutritional program and policy interventions.

They are like cash distributed because of cash transfer policy etc. So, lastly, this is used to identify the intervention of certain programs maybe as nutritional supplements how this has impacted the larger population. We can understand the implications of policy designs or policy interventions.

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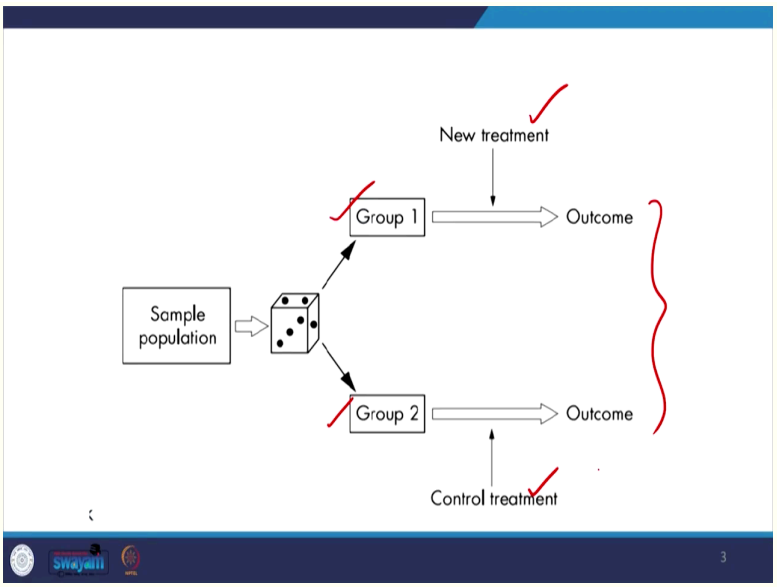

So, this is defined as what is our sample and how exactly this sample sampling frame is defined. There are 2 groups defined from the population: one is the treatment group another is called the control group and then both the case we will have certain outcomes and that outcome has to be compared.

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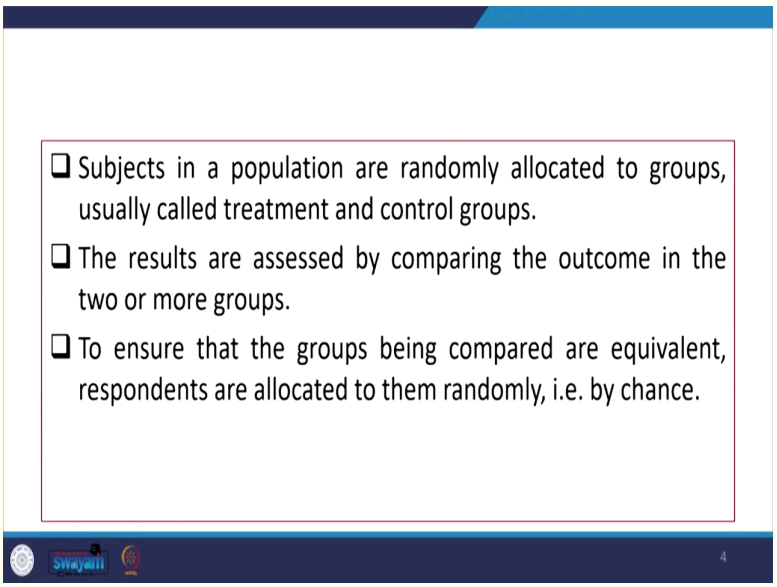

Subjects in a population are randomly allocated to groups usually called treatment and control groups. The results are assessed by comparing the outcome in the two or more groups. To ensure that the groups being compared are equivalent, respondents are allocated to them randomly.

The numbers allocated to both groups should be randomly allocated. There should not be any single biasedness. So, that means, those are present in each of the groups by chance.

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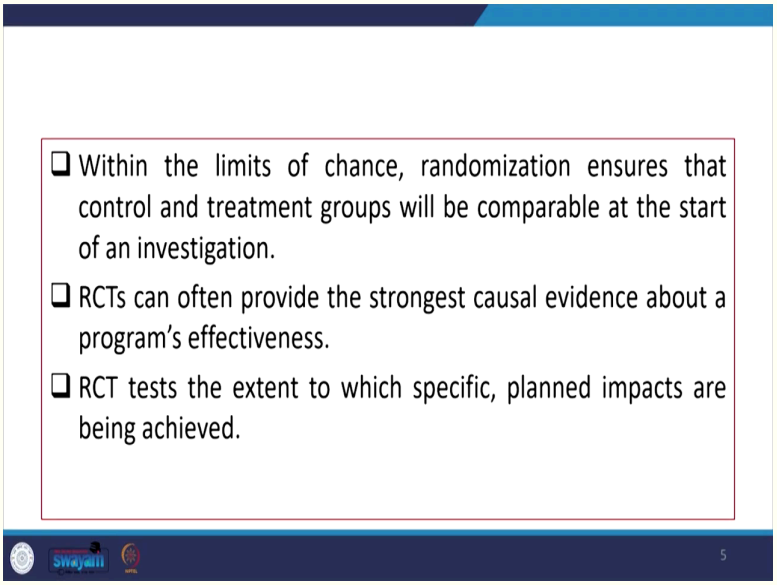

Within the limits of chance, randomization ensures that control and treatment groups will be comparable at the start of an investigation. RCTs can often provide the strongest causal evidence about a program's effectiveness. RCT tests the extent to which specific, planned impacts are being achieved.

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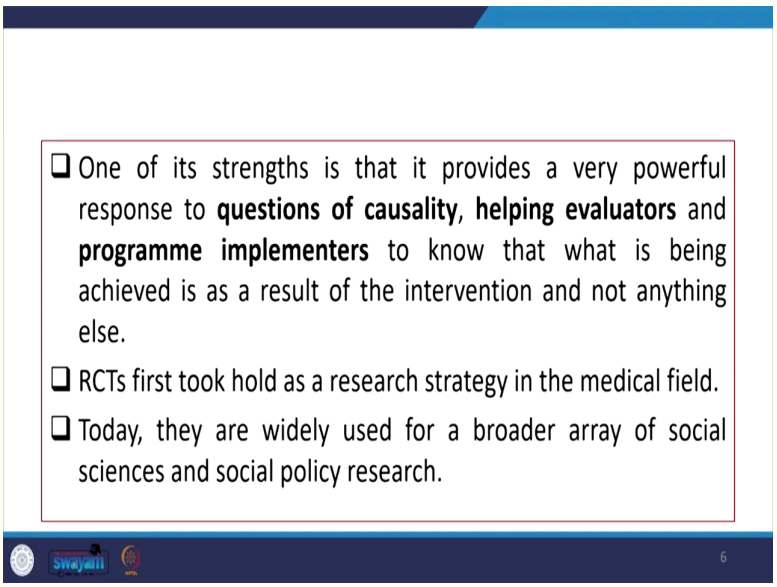

One of its strengths is that it provides a very strong or significant response to questions of causality and helps evaluators and programme implementers.

So, this provides the cause-effect relationship and helps the evaluator as well as the programme whoever is trying to implement. And this helps us to know that what is being achieved is because of the intervention and not anything else. RCTs first took hold as a research strategy in the medical field. They largely apply RCT s design. Today, they are widely used for a broader array of social sciences and social policy design as well.

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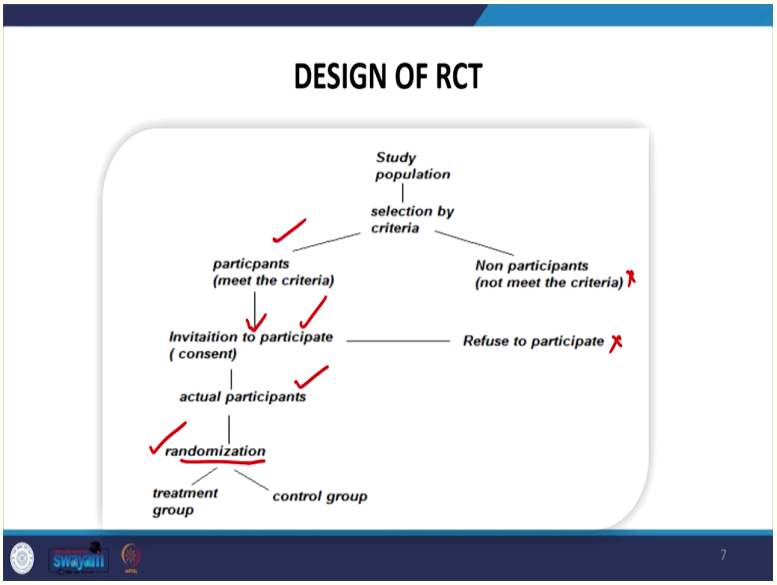

The design of RCTs is as follows: starting with the population, then there are selection criteria. One is called participants and the other is called non-participants. So, participants who meet the criteria and non-participants are not at all part of the program.

We are considering the population who are eligible for cash transfers. Then within the eligible group, we have to define the experimental group and non-experimental group.

So, those who meet the criteria of the cash transfer program are considered. Then invitation to participate some may refuse, they are also subtracted. Their consent should be taken thus it was important this has to be followed.

If they are not relevant to us, then we go to actual participants who are relevant to us. Then we will divide into control groups and treatment groups and that is possible through a

randomization technique. So, when perfect randomization is understood; that means, we are actually following the design of RCT correctly.

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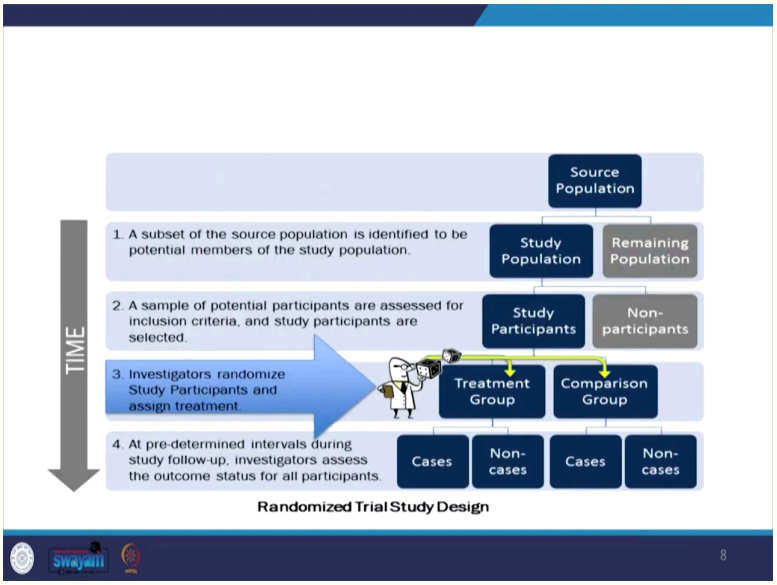

So, now comes the time for allocation in the randomized trial study design. First, a subset of the source population is identified to be potential members of the study population and there could be a source population could be the study population and the remaining population. Second a sample of potential participants is assessed for inclusion criteria and study participants are also selected.

So, accordingly, a study participant and non-participants could be understood. We can subtract those who are not actually the participants. Then investigators randomize study participants and assign the treatment. Now, we can compare the treatment group and then we can have a comparison group.

At predetermined intervals during study follow up and investigators assesses the outcome status of all participants. Then further treatment groups and comparison groups are divided into cases and non-cases. Then further stratification can also be made, further design can also be made to compare the results.

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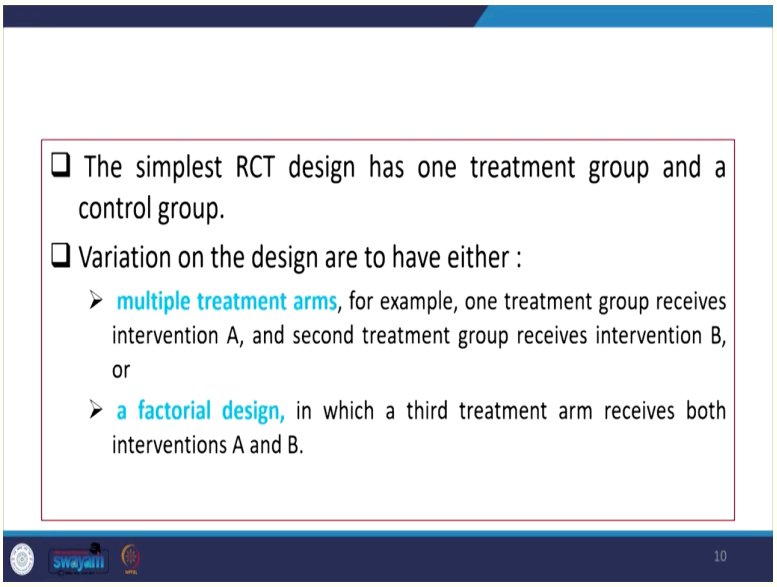

The simplest RCT design has one treatment group and a control group. A variation on the design is to have either: there will be multiple treatment arms or there will be factorial design.

In the case of multiple treatment arms, for example, one treatment group receives intervention A, and the second treatment group receives intervention B, accordingly, we can classify. In the case of factorial design in which a third treatment arm receives both intervention and intervention B.

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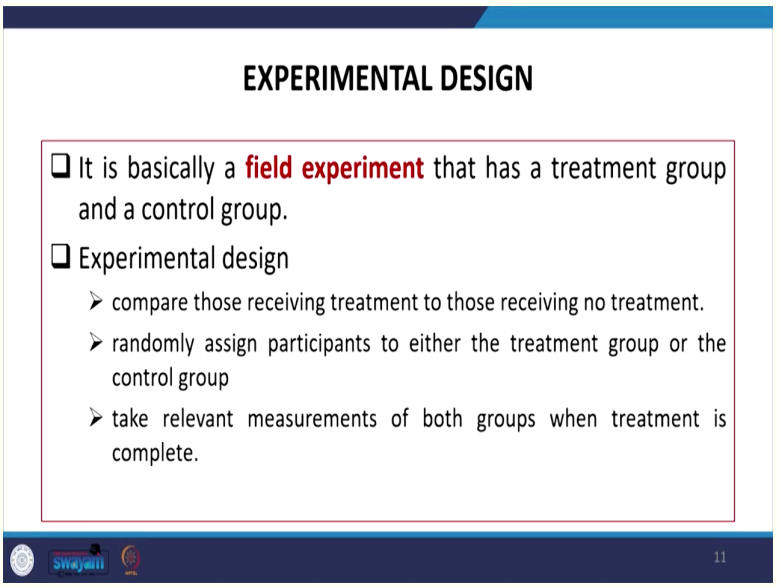

Coming to the experimental design further, it is basically a type of experiment that has a treatment group and a control group. In the case of experimental design, we need to compare those with the receiving group to those receiving no treatment. We have to also make sure that the case observations are randomly assigned, in both groups and we need to take relevant measurements of both the groups when treatment is complete.

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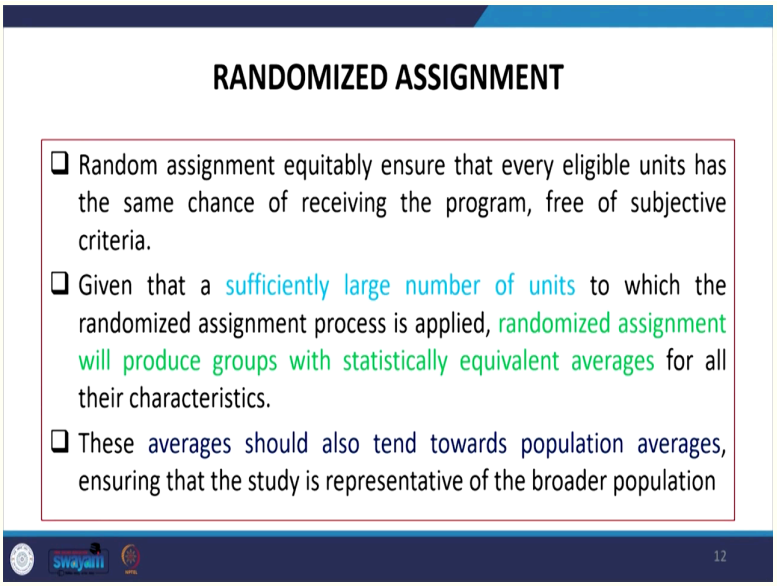

Then what do you mean by randomize assignment and what is the focus of the RCT tool, RCT design? So, randomization is quite important. So, random assignment equitably ensures that every eligible unit has the same chance of receiving the program, free of subjective criteria or any sort of reservations has to be avoided.

It is a randomized assignment given that a sufficiently large number of units to which the randomized assignment process is applied. The randomized assignment will produce groups with statistically equivalent averages of all their characteristics.

These averages should also tend toward population averages, ensuring that the study is representative of the broader population. So, there might be some issues here like if your average treatment has not been experimented with in different groups or different populations, larger population your result might not be applicable to a larger context. We have to make sure that as well.

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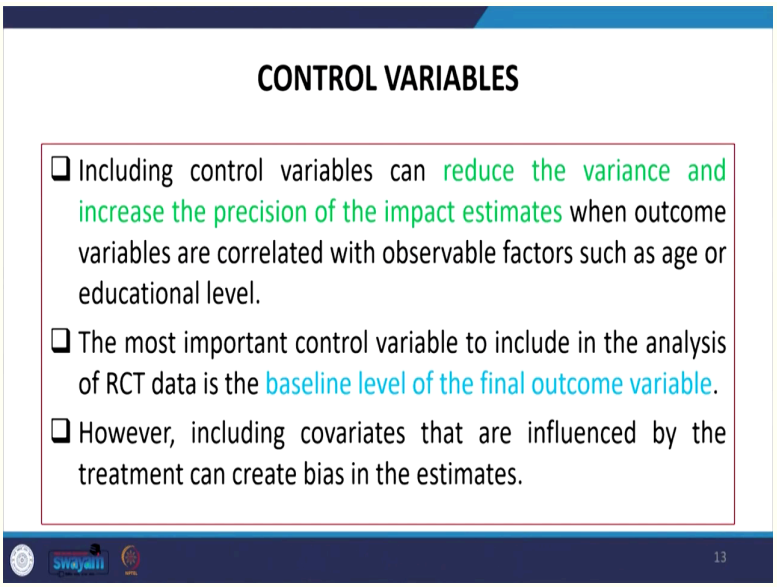

So, what about the control variables, this includes the variables that can reduce the variance and increase the precision of the impact estimates when outcome variables are correlated with observable factors such as age or education level. In that case, control variables are important with this helps in reducing the variance and increasing the precision of the impact estimates.

The most important control variable to include in the analysis of RCT data is the baseline level of the final outcome variable. So, the baseline level of the final outcome variable is most important to emphasize. However, including covariates that are influenced by the treatment can create bias in the estimates.

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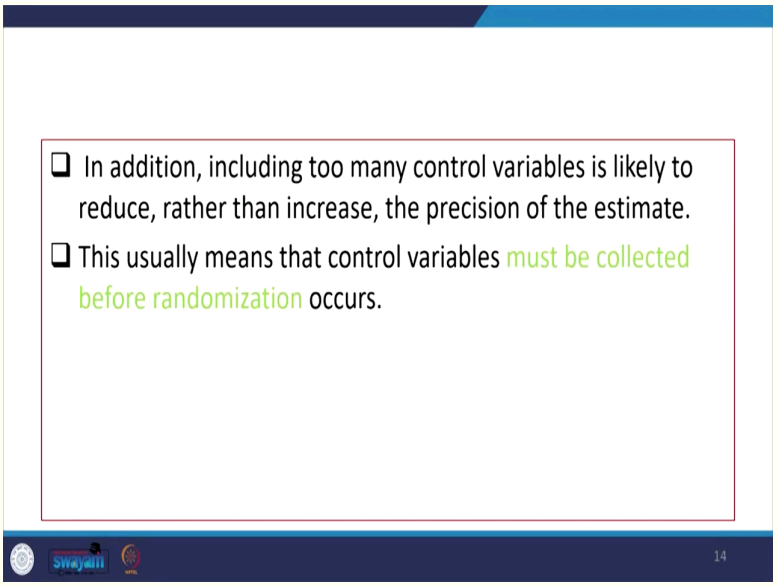

In addition, including too many control variables is likely to reduce, rather than increase the precision of the estimate. So, too many layers have to be taken care of in this RCT design. This usually means that control variables must be collected before randomization occurs.

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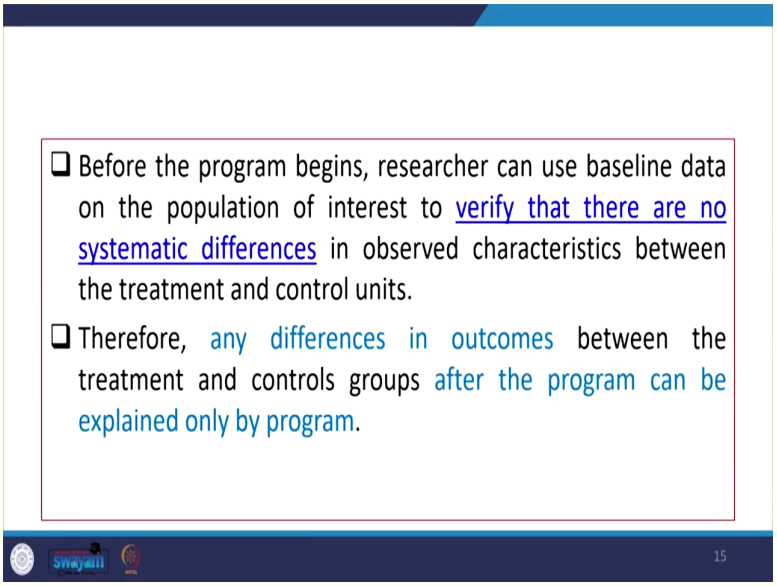

So, before randomization, those control variables must have been thoroughly observed. Before the program begins researcher can use baseline data on the population of interest to verify that there are no systematic differences in observed characteristics between the treat treatment and control units. Therefore, any differences in outcome between the treatment and control groups after the program can be explained only by the program itself.

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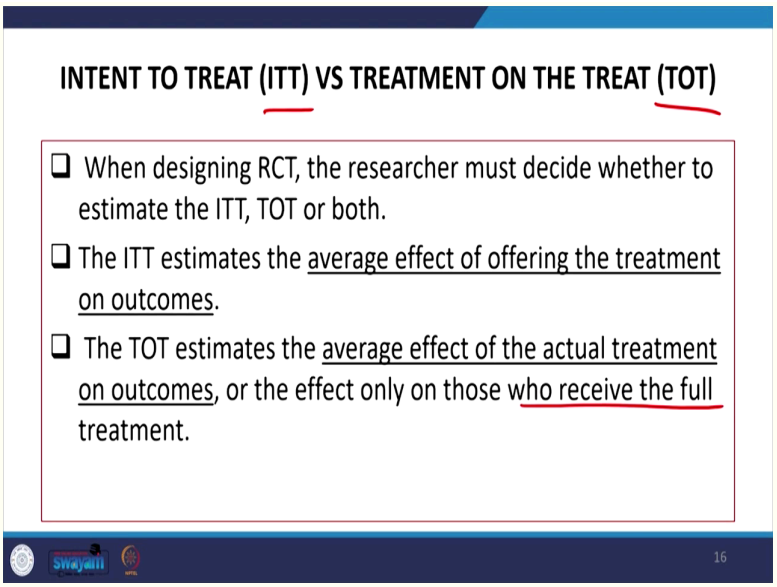

Now, coming to certain aspects like called intent to treat ITT versus treatment on a treat that is called TOT. So, we are explaining called ITT and TOT, when designing RCT the researcher must decide whether to estimate the ITT or TOT. So, that is on treat treatment on the treat and intend to treat.

So, ITT and TOT or both the ITT estimate the average effect of offering the treatment on outcomes, this is basically the average effects regarding the treatment. The TOT treatment on the treat, those are the treat treatment group the treatment on the treat is estimated. TOT estimates the average effect of the actual treatment of on the outcomes, or the effect only on those who receive the full treatment.

So, for those who receive the likewise in a PSM we discuss about an ITT as compared to ITE here also there is a difference between ITT and then TOT. TOT is specifically on the group, where the actual or full treatment is taken or given.

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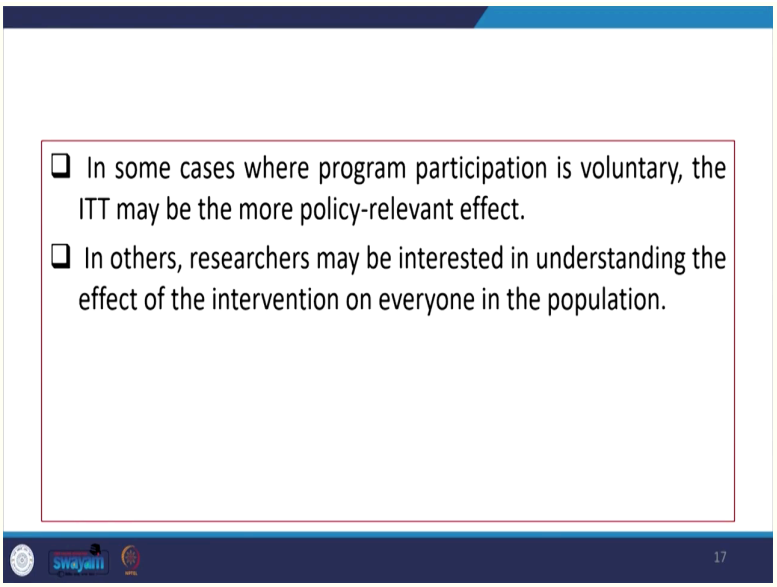

In some cases where program participation is voluntary, the ITT may be the more policy-relevant effect. Like if program participation is quite voluntary instead of it is specifically targeted.

When it is voluntary that means, there are huge randomness attached. So, it is better to evaluate both the groups when it is involuntary or very specific then you have to differentiate who are the specific persons willing to take the program and how what really happened to them. So, researchers may be interested in understanding the effect of the intervention on everyone in the population. So, in that case, the ITT is maybe ITT may be considered.

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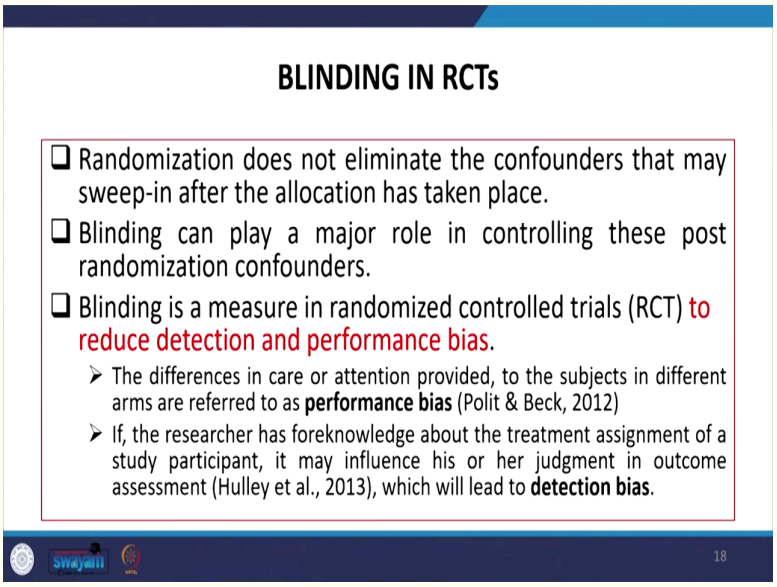

What about blinding in RCTs. Blinding in RCTs is discussed as like randomization does not eliminate the confounders that may sweep-in after the allocation has taken place. Though the confounding variables, the confounders may have also impacted they might create some blinding on the results. So, blinding can play a major role in controlling these post-randomization confounders.

So, even if you have done certain randomization with the assumption that there is no difference between these two groups, but still due to the confounders the control variables might have actually impacted. So, blinding plays an important role. Blinding is a major in randomized control trials. So, blinding is a major in RCTs to reduce detection and performance bias.

The differences in care or attention provided to the subjects in different arms are referred to as performance bias. If the researcher has foreknowledge about the treatment assignment of a study participant, it may influence his or her judgment in outcome assessment, which will lead to some detection bias. So, if there is some prior understanding that may lead to certain detection biases those could be also identified through the RCT design.

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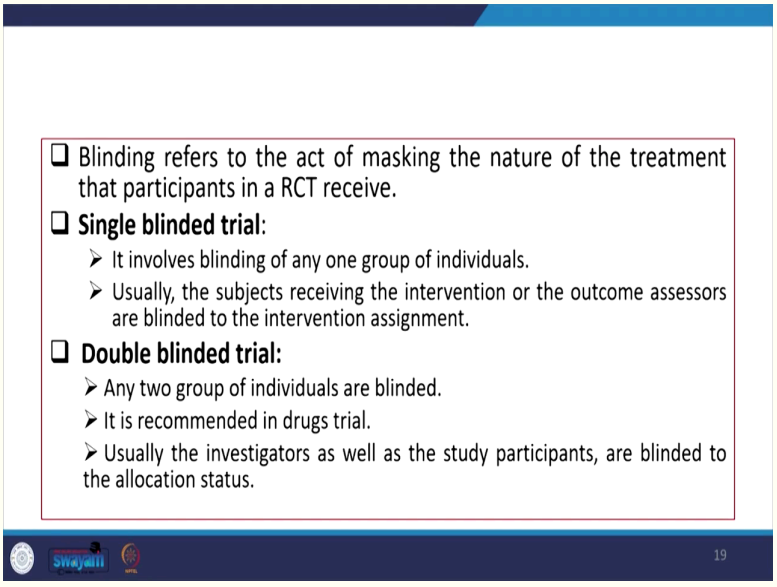

Blinding refers to the act of masking the nature of treatment that participants in an RCT receive. Single blinded trial: it involves blinding any one group of individuals. Usually, the subjects receiving the intervention or the outcome assessors are blinded to the intervention assignment.

What do you mean by then that is basically called a single-blinded trial and as compared to the double-blinded trial. Single blinded 1 where this involves blinding of any one group of individuals. Whereas, in the case of a double-blinded trial where any 2 groups of individuals are actually blinded. In case of single usually, the subjects receiving the intervention or the outcome assessors are blinded to the intervention assessment.

So, the; so these two difference has to be noted very carefully, but in case of a double-blinded one it is recommended in a usually in a drugs trial, wherever drugs trials are taken both have been actually blinded and they do not have any sort of information about the kind of intervention is going to be given. Usually, the investigators as well as the study participants are blinded to the allocation status. Who has been allocated nobody knows.

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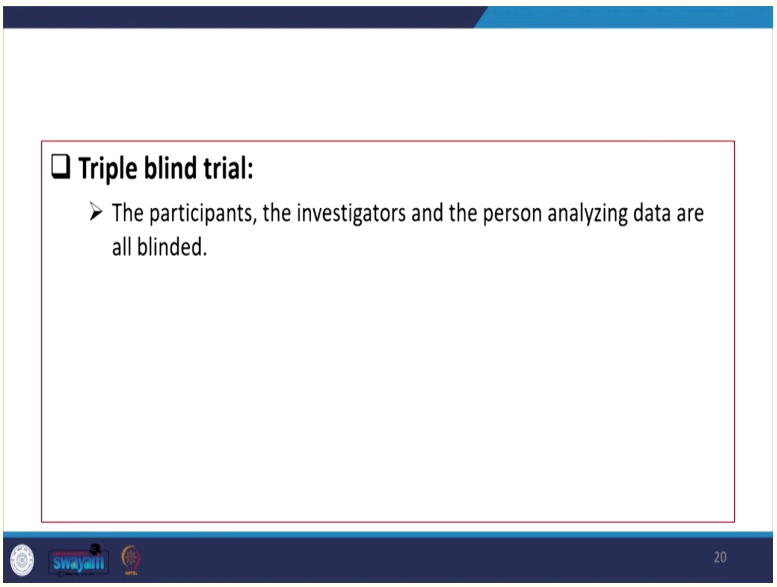

There are triple-blinded trials as well. The participants, the investigator, and the person analyzing the data are all blinded. It is not about the participants, it is about the investigator also.

So, what is actually being a being run or whoever are analyzing these context they also do not know. So, finally, there might have been some codes assigned to the person, so which code carries for what purposes nobody knows, but we can an accordingly get the result and comparison is made you, so then this must be very complicated for sure. So, then now you might have understood how randomization is done.

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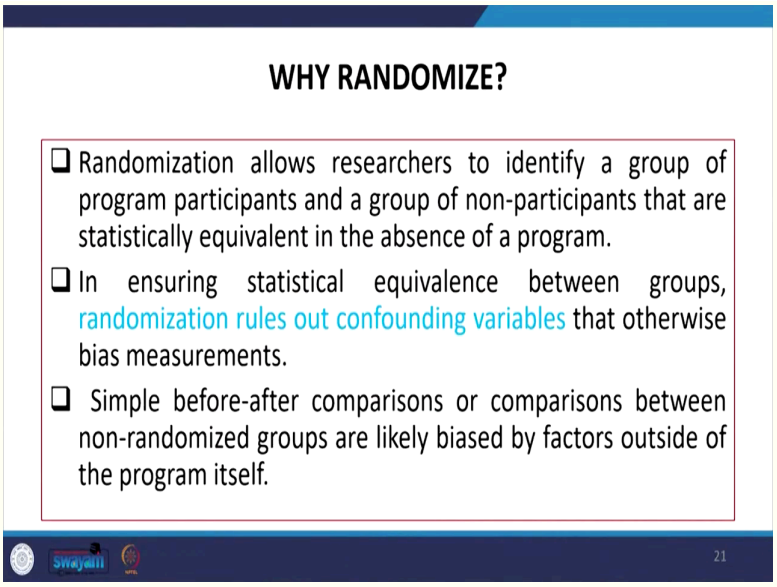

So then, why to randomize? It is quite obvious, that we will understand the nonbiased component, in the experimentation. So, the randomization allows researchers to identify a group of program participants and a group of nonparticipants that are statistically equivalent in the absence of a program. In ensuring statistical equivalence between groups randomization rules out confounding variables.

So, this will rules out confounding variables and also statistical equivalent results without or with the absence of a program can also be compared. Simple before-after comparisons or comparisons between nonrandomized groups are likely to likely be biased by factors outside the program in itself; that is where randomization is needed. So, all these three points regarding randomization are equally important to note.

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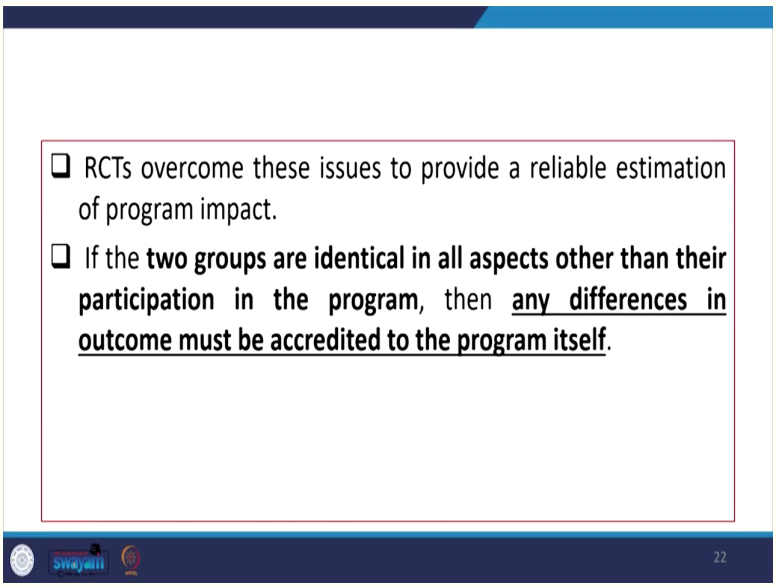

RCTs overcome these issues to provide a reliable estimation of program impact. If the two groups are identical in all aspects other than their participation in the program, then any differences in outcome must be accredited to the program itself.

If they are completely identical in all aspects and they are also randomized equally, then any kind of outcome is derived. Obviously, would be resulted due to the program itself which is why randomization is needed.

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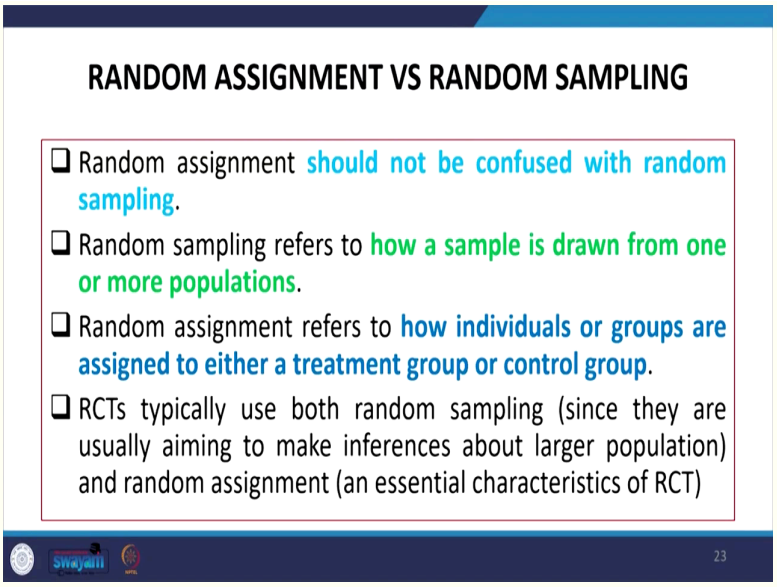

Random assignment versus random sampling, what do you mean by that. So, it is an obvious question that should be verified. So, is it the random sampling that is called random

assignment we need to clarify? Random assignment should not be confused with random sampling. Random sampling refers to how a sample is drawn from one or more populations.

Whereas random assignment refers to how an individual or a group are assigned to either a treatment group or to a control group. So, basically, it is not just the persons to be randomly picked up rather we are actually dividing into two groups. How they have been categorized if that is followed with a random assignment without having any bias that is basically called a random assignment.

RCTs typically use both random sampling, since they are usually aiming to make inferences about larger populations and random assignment. So, both random sampling and random assignment because, of essential characteristics of a RCT. So, RCT as well as random sampling both are used in RCT design, because of making or result more populated or more representative.

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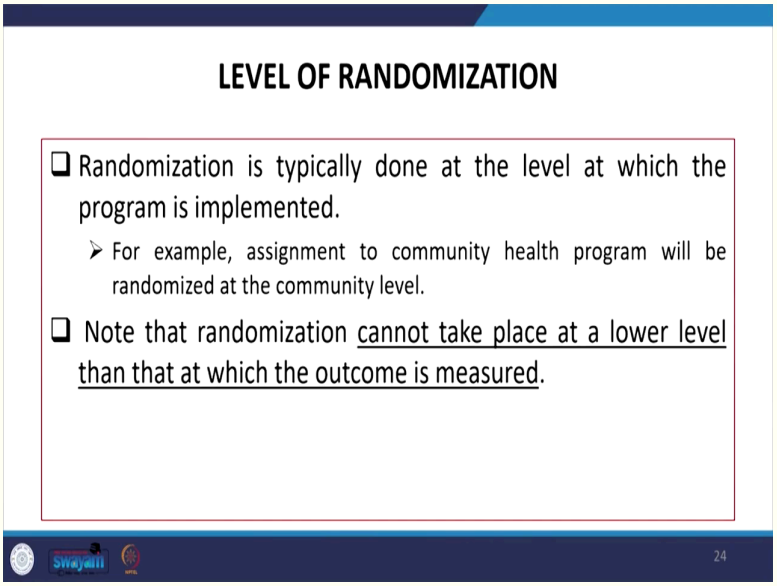

So, what do you mean by what is the level of randomization? Randomization is typically done at the level at which the program is implemented. So, for example, an assignment to a community health program will be randomized at the community level, if it is at the household level or at the district level.

Then accordingly district-level randomization is made. Note that randomization cannot take place at every lower or disaggregated level; because there are would be lots of possible biasedness.

So, usually, all kind of program evaluation technique is taken by the J-PAL group, J-PAL group in their maximum program evaluation techniques, usually, do it at the district level. So, if it is lower than that of the community level, then it is it might not find the randomization appropriately.

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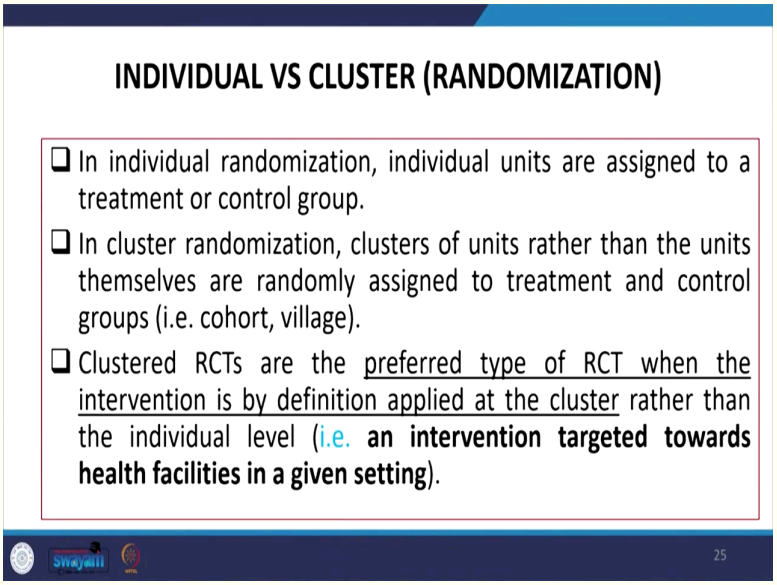

Then individual versus clusters how randomization is made. In individual randomization, individual units are assigned to a treatment or control group. In cluster randomization, clusters of units rather than the units themselves are randomly assigned to treatment or group.

So, basically, we know that clusters like cohort study are made. So, cohort, once we are defined the cluster itself are selected instead of any person within the cluster, are selected to both the groups. A cluster may be a village, likewise in an NSS in census data cluster villages we are defined.

So, accordingly, the randomization is done. Cluster RCTs are the preferred type of RCTs when the intervention is by definition applied to the cluster level rather than at the individual level. So, that is an intervention targeted toward health facilities in a given setting.

If it is a cluster base a given setting is given how health intervention is actually implicated at a community level or at a cluster level. The statistical power in cluster RCTs is typically lower than that of the individual RCT.

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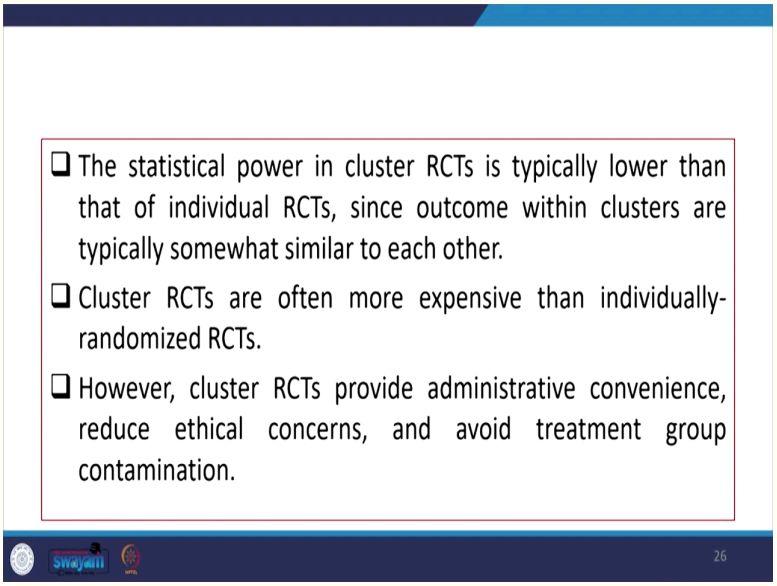

So, that has to be noted down. Since outcomes within clusters are typically somewhat similar to each other. So, the differences cannot be actually identified across the clusters, but across the individual, the cluster impact of RCTs or any program can be identified. Cluster RCTs are often more expensive than individually randomized RCTs. These are more expensive as well. However, cluster RCTs provide administrative convenience. And reduce ethical constraints and avoid treatment group contamination.

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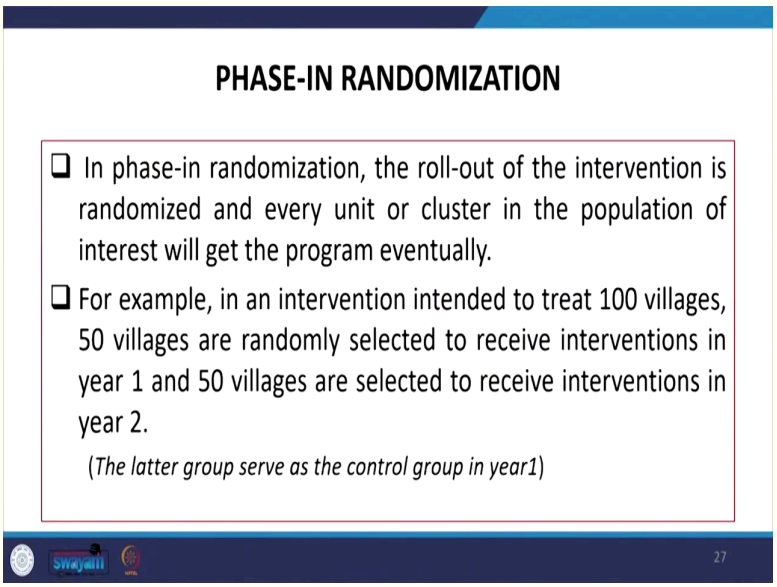

What do you mean by phase-in phase -in randomization? In phase-in randomization the rollout of the intervention is randomized and every unit or cluster in the population of interest will get the program eventually. For example, in an intervention intended to treat around 100 villages, 50 villages are randomly selected to receive interventions in year 1st and 50 village is selected to receive intervention in 2nd year.

And then a comparison can be made. The latter group that is in the surveys is the control group in year 1. Phase-in designs also reduce concerns of in inequity and provide incentives to maintain contact.

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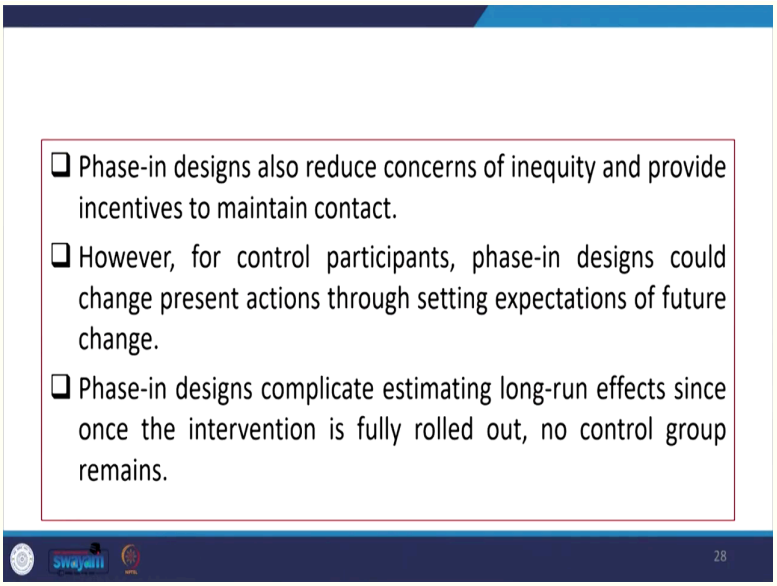

However, for control participants, phase-in designs could change present actions by setting expectations of future change. Phase-in designs complicate estimating long-run effects since once the intervention is fully rolled out, no control groups remain.

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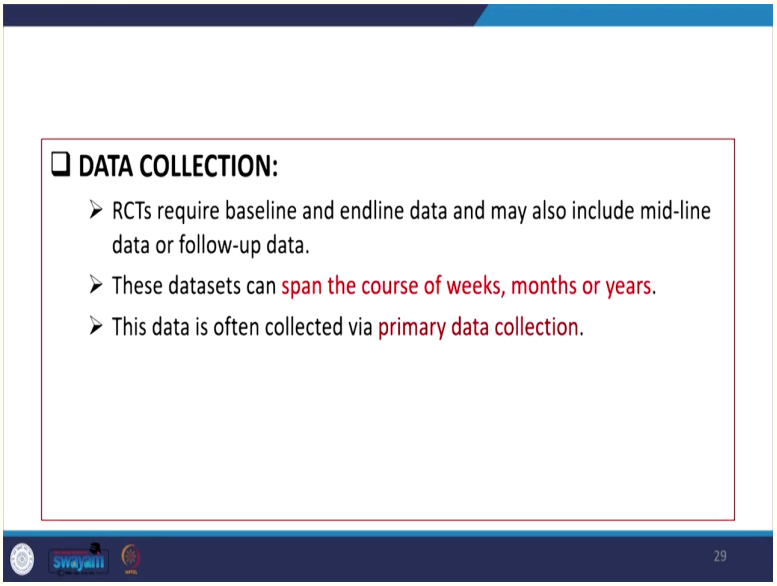

So, regarding data collection RCTs require baseline as well as end-line data and may also include mid-mid-line data, some in-between data is required to have proper follow-up information for better designing in the second one or for better implementation of the RCTs. These datasets can span the course of weeks, months, or years. This data is often collected via the primary data collection method.

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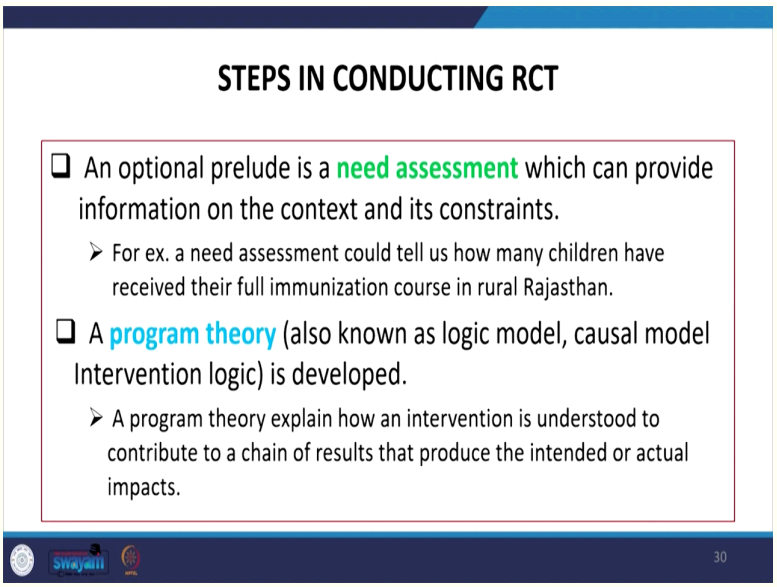

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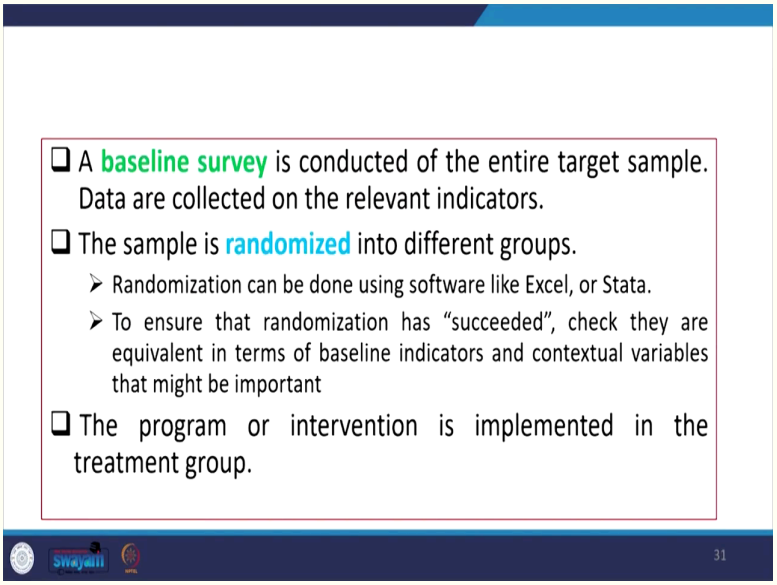

So, there are steps involved in conducting RCTs. Steps like one is like need assessment, program theory, baselines survey, randomization techniques, monitoring, some follow-ups. So, etcetera is involved in case of need assessment and this is an optional prelude. An optional prelude is a need assessment, which can provide information on the context and its constraints.

That is as an example need assessment could tell us how many children have received their full immunization course in rural Rajasthan if focus is Rajasthan. How many have received.

So, that means, if we wanted to know about the number of children, then need base assessment is made.

A program theory is also known as a logic model, causal model intervention logic is actually developed as a program theory explain how an intervention is understood to contribute to a chain of results that produce the intended or actual impacts.

A baseline survey is conducted of the entire target population or sorry entire target sample; data are collected on the relevant indicators. A sample is all samples should be randomized into different groups as we already mentioned. Randomization can be done using software like Excel or Strata, to ensure or randomize tables as well.

To ensure that randomization has succeeded and check they are and check that they are equivalent in terms of baseline indicators and contextual variables that might be important in the study. The program or intervention is implemented in the treatment group.

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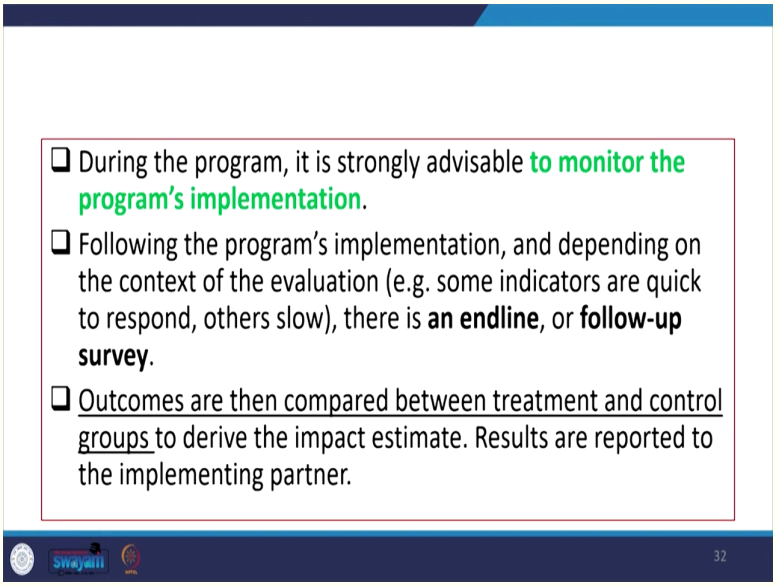

During the program, it is strongly advised to monitor the programs implementation. So, monitoring is always essential in program implementation design or program evaluation design, following the implementation of the program and depending on the context of the evaluation, end line and follow-up survey should be conducted.

Some indicators are quick to respond and others may slow; so, intervention is going to give you the right direction. Outcomes are compared between treat treatment and control rules to

determine the impact estimate. Results are reported to the implementing partner, once this process have been completed.

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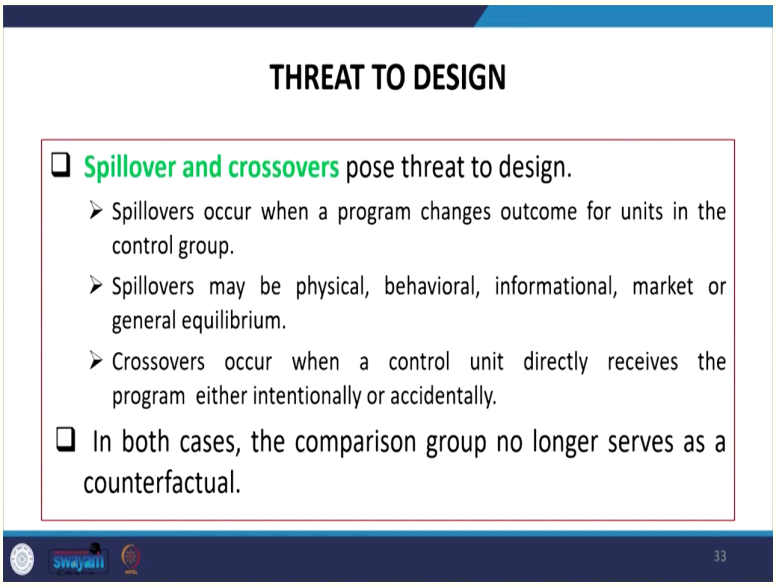

So, then a couple of things to be guided to you to understand this very correctly. We are just coming to an explanation of the threat to design. To design how to read at the design correctly. First, one is called spillover and crossover poses a threat to design.

There are some spillover and crossover issues. Spillover occurs when a program changes outcomes for units in the control group. If that program gets changed and has some outcomes in the control group then, the spillover impact may create some problems.

Spillovers may be physical, behavioral, informational, market or general equilibrium based. Whereas, a crossover occurs when a control unit directly receives the program either intentionally or accidentally by any reason, if there are some crossover, then it is very difficult to go by the RCTs. In both cases, the comparison groups no longer serve as a counterfactual. So, they are no longer a counterfactual, where the treatment cannot be actually evaluated.

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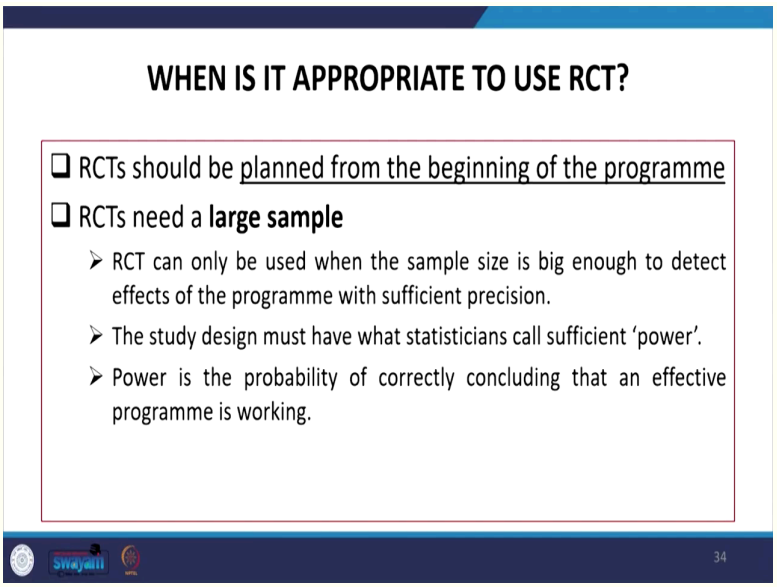

So, when is it appropriate to use RCT? This is very interesting to be noted, RCT should be planned from the beginning of the program. The entire structure of the RCT is very essential when going for RCT based evaluation.

RCT RCTs need a large sample. This is another important aspect. This can only be used when the sample size is big enough to detect the effects of the program with sufficient precision. The study design must have what statisticians call sufficient 'power'. Power is the probability of correctly concluding that an effective program is In fact, working.

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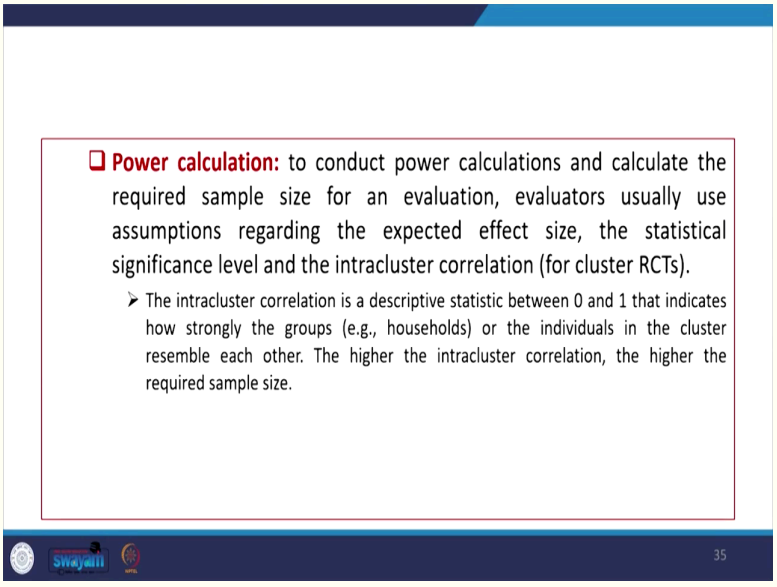

Then again we need to understand what you mean by a power calculation. This is basically to conduct and calculate the required sample size for any evaluation. Evaluators must use assumptions regarding the expected effect size and the statistical significance level and the intracluster correlations.

If you have any sort of cluster design, cluster RCT design. Intracluster correlations is basically a descriptive statistic between 0 and 1 that indicates how strongly your groups are household base or if it is individuals in the clusters resemble each other in both the groups of comparison.

The higher the inter intracluster correlation the higher is the required sample size to be considered for RCT design. RCT should be undertaken following formative research or evaluation RCT must be appropriate to the nature of the program being assessed.

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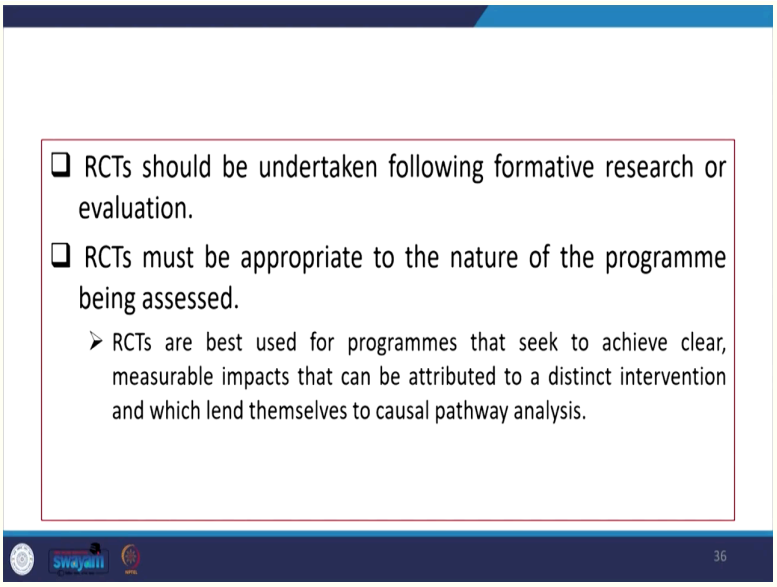

RCTs are best used for programs that seek to achieve clear measurable impacts that can be attributed to a distinct intervention and which lend themselves to a certain causal pathway for better analysis in case of program implementations.

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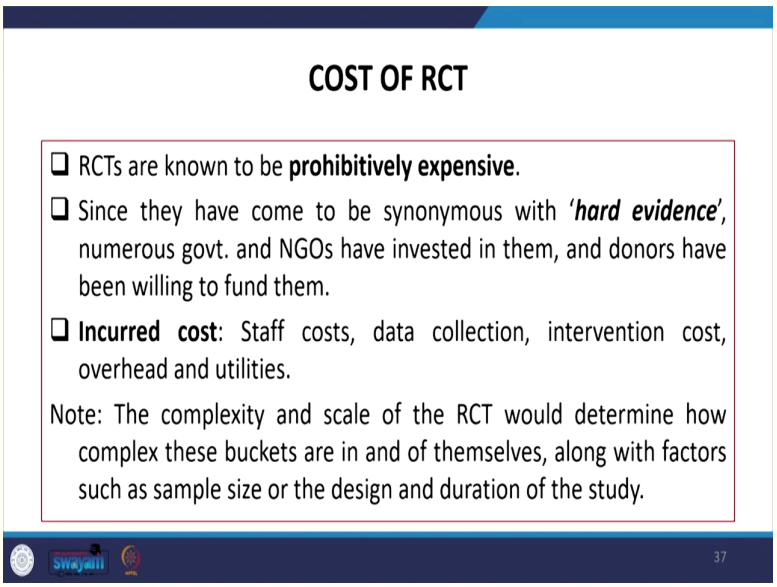

A couple of issues at the last are very essential like understanding cost of RCTs. How the expenses are born has to be decided, because you are evaluating comparing groups and the programs whether are received by them or not. And there are so many interventions as well like behavioral as well that may have certain crossovers.

So, cost is quite essential. RCTs are known to be the prohibitively expensive method. Since they have come to be synonymous with hard evidence, numerous government and NGOs have invested in them and donors have been willing to fund them because this they give concretized information.

This incurred cost, because because of staff costs, data collection method, intervention cost, overhead cost, utilities the time lag as well. The complexity and scale of the RCTs would determine how complex these buckets are in hand and of themselves along with factors such as sample size the design as well as the duration of the study.

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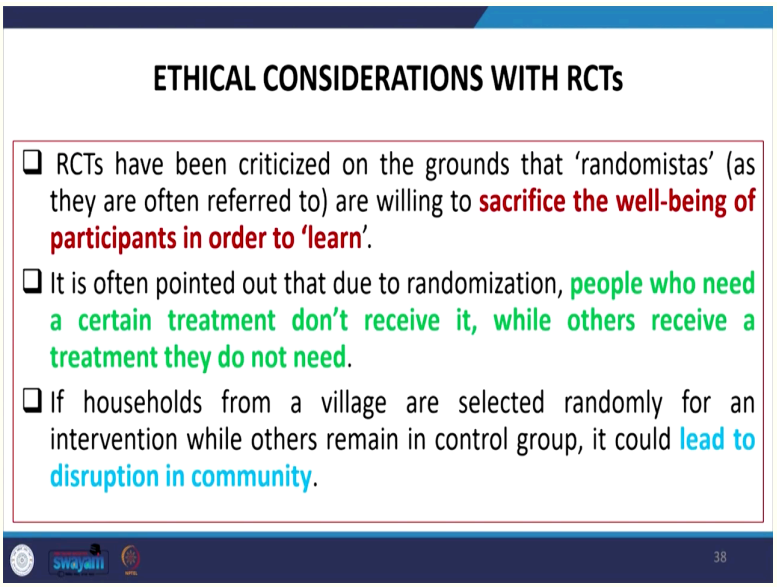

Nonetheless, we should have ethical considerations, because we are direct observation observing the persons or the clusters. So, their ethical considerations should have been taken, there should not be any sort of conflicting or competing interests. Their identities are at stake. So, some ethical considerations should be taken beforehand. RCT has been criticized on the ground that random 'randomizes' they are also often referred to as like referred to as this what a 'randomized' are willing to basically sacrifice the wellbeing of participants in order to learn.

It is often pointed out that due to randomization, people who need a certain treatment do not receive it, while others have a treatment they do not need. So, that is very interesting. So, some do not need and wherein some cases they have got it, but they did not have need.

And in some cases they received it, they did not receive it even. If households from a village are selected randomly for intervention, while others remain in control group. It could lead to disruption in the community, because some may assume that why they are selected, why and I am not selected. There might be the possibility of further conflicts and violations within the community.

So, after explaining all sort of arrays of for the chain of discussion in RCT, we have discussed about its design, its steps, its randomization technique, its ITT and TOT, and its cost context, then an ethical consideration at the end is always discussed with this I am quite sure that you might have received huge understanding related to RCTs. Further details on it we are going to add in our next episode, not on this call.

It will be discussed in our next year maybe. This time we have clarified the basic design of RCT, its advantages, their disadvantages, and their limitations. The practical side of applying to a data, it requires a direct field observation and it involves cost. Therefore, we are also limited in explaining all those aspects.

So, if you have any queries do not hesitate and we will try our best to address to you through our team, their Mister Milind and Mister Komal will be also proactively helping in this regard to get a direction in this design for the program evaluation or evaluation-based methods. With this, I think it is time to stop and we will explain further details in the next class.

Thank you.