Supplementary Online Content


**Trial Protocol**

This supplementary material has been provided by the authors to give readers additional information about their work.
## Protocol Title:

**IMPROVING OUTCOMES WHILE REDUCING COSTS IN HIGH-RISK CHILDREN: THE HOUSTON PEDIATRIC QUALITY PROJECT**

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## Co-Investigators:

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## Project Coordinator:

To be determined

## Population:

Children identified as ‘high-risk’

## Number of Sites:

Single site – UT Houston and Children’s Memorial Hermann

## Project Duration:

The length of the project will be 2-6 years.
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**Background:** For patients of all ages, there is a pressing need to both improve outcomes and reduce costs. Improvements in care of high-risk children can result in the greatest gains in healthy life years and the greatest savings in long term costs. Dr. Guy Clifton has conducted a review to explore which children in the population treated at UT Health have the greatest health care cost. Among 3240 children with Medicaid funding from Amerigroup (our predominant Medicaid insurer) and treated by our faculty, 234 high-risk children - ~7% of all beneficiaries ---accounted for 45% of total spending after the neonatal period. Nearly three-quarters of the expenditures resulted from rehospitalizations. The greatest expenditures were for the sequelae of prematurity. The next 3 most expensive problems were cardiac anomalies, chronic behavioral or neurologic problems, including recurrent seizures, and reactive airway disease. A broad variety of chronic problems accounted for the remaining expenditures.

We will conduct two projects to improve the outcomes and reduce the costs of these children. The first involves extremely premature infants (<29 weeks gestation) during the first 12 months after birth. The second involves other high-risk children of any age.

This proposal describes the second project. Both projects involve assessing the costs and benefits of providing comprehensive, coordinated health care in a patient-center medical home. A small team of dedicated care givers will be available to these children and their parent(s) at all hours either in the clinic or by telephone. The hypotheses and the methods are based in part on the success achieved in studies of medical homes in diverse age and disease groups¹ and in a large randomized trial of comprehensive follow-up care for high-risk infants co-directed by Dr. Tyson in Dallas and published in JAMA.² As in the two projects we will conduct, comprehensive care included care for both acute and chronic illness provided by a small team of highly committed caregivers available in the clinic or by phone at all times.

This trial showed that comprehensive follow-up care reduced life threatening illness (death or pediatric ICU [PICU] admission) in these infants by more than 40% while their costs of care were a third lower than with conventional care (as estimated by health care economists at the UT Houston School of Public Health using state-of-the-art methods).

**Funding:** These projects will be supported in part by Texas Health and Human Services and hopefully funding from foundations or federal sources. A portion of any savings in Medicaid expenditures of Amerigroup will be returned to the Medical School. If this project is rigorously conducted and successful in confirming the hypotheses below, it will help to establish a sustainable model of payment and delivery of cost-effective care for all high-risk children that can be used in academic medical centers throughout the U.S.

**Primary Hypothesis:** Compared to usual care, comprehensive care as described below will be cost effective in reducing serious illness (illness that results in death, PICU admission, or prolonged hospital stay (>median) for high-risk children during the first year or longer after enrollment in the program. The program will be considered cost effective if

1. it reduces serious illness without increasing total estimated costs of care (healthcare system perspective);
2. it reduces estimated total costs without increasing serious illness or
3. as we expect, it reduces both serious illness and total estimated costs.

**Secondary Hypotheses:** During the first year or longer after enrollment in the program, comprehensive care will
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1. Reduce serious illness (primary medical outcome) by >33%;
2. Reduce total pediatric ICU days by >33%;
3. Reduce total hospital days by >15%;
4. Reduce emergency department (ED) visits by >33%;
5. Reduce the above measures to the greatest extent among children of mothers with limited education (with a significant interaction between treatment group and maternal education);
6. Double the proportion of parents highly satisfied with their care;
7. Reduce estimated total costs as assessed from a variety of perspectives (government [Medicaid costs], medical school, hospital, parents, and society). Health care system costs (primary economic outcome) and Medicaid costs are both hypothesized to be reduced by >25%.
8. Increase Medical School revenues relative to costs.
9. Will have greater benefits in the 2nd half of the project than in the 1st half as caregiver experience and understanding of how to best prevent serious illness.

Population:

Inclusion criteria: The patients involved in this project will be children who based on past experience and the judgment of the investigators are expected to have a high likelihood of hospitalization and high medical costs. Though most children enrolled will not have private insurance, children of any payer status will be selected based on their diagnoses, prior hospitalizations, and social risk factors. Based partly on Dr. Clifton’s review, we would include children who had an ICU admission, a prolonged hospitalization (>14 days), or two or more hospital admissions in the prior two years for treatment of congenital heart disease or other major congenital anomalies, recurrent seizures or other neurologic disorders, asthma or other chronic pulmonary disease, short gut syndrome or other gastrointestinal disorders, diabetes or other major endocrine disease, or other chronic disorders, particularly if they have social risk factors that increase their risk (single parent, limited maternal education, lack of private insurance).

Exclusion criteria: We will not enroll children whose parent(s) want the primary care physician to be a private practitioner. After enrollment, the mothers of occasional patients may choose to seek care from other physicians. Because of the special attention provided those in the comprehensive care group, attrition would be expected to occur most often in the usual care group. We would not drop these children from the project but continue to assess the care, outcomes, and costs as allowed with the consent obtained prior to project entry. Such data collection is needed to allow intent-to-treat analyses, ensure baseline comparability of the two treatment groups, avoid selection biases in loss to follow-up, and assess all consequences of the two approaches to care at UT Health (including the decision of some parents [hopefully very few] to seek care elsewhere.).

Recruitment: Participants will be identified using a variety of methods. At the start of the project, we will identify potential participants:

1. Children with the highest faculty billings in the prior 2 years; and
2. Children who have any of the above diagnoses and high expected medical costs currently followed in pediatric specialty clinics (e.g., CHOSEN clinic; pulmonary clinic; and other specialty clinics), particularly if they have not been treated by UT Health physicians for less than two years (and thus may not be identified as potential participant from total faculty billings).
3. Children born at ≥29 weeks gestation discharged from the MHH neonatal ICU or term nursery who do not qualify for the High-Risk Infant Clinic but have conditions (e.g., chronic lung disease or major congenital anomalies) expected to result in a high likelihood of rehospitalization;

4. Older children discharged from MHH who have had a PICU admission or hospitalization ≥14 days and have one or more diagnoses associated with a high likelihood of rehospitalization and high medical costs;

5. Children with such diagnoses and high expected medical costs who reach an age (18-24 months) when they can no longer qualify for the High-Risk Infant Clinic;

6. Children with high costs identified from periodic tallies of Pediatric faculty billings in the past year who have not otherwise been identified.

**Design:** Patients will be stratified into two groups according to whether or not they are considered to have greater than median risk of repeated hospitalizations for patients in this study as judged from their diagnosis, prior hospitalizations, and maternal education. This approach is used to help ensure that the two treatment groups will have a similar proportion of patients at very high risk. Some judgment will be required in assessing the appropriate risk stratum. To avoid bias, these decisions will be made without knowing the treatment group that would be received.

Children will be randomized to either usual care (the care that would be provided in the absence of this project) or comprehensive care. Those who refuse consent will receive usual care. Treatment assignment will be randomized because:

1. Provision of comprehensive care for all high-risk children is not feasible and not affordable with the limited current reimbursements for the care of these children and the funds, resources, and personnel that we have been able to obtain.

2. Under the ethical principle of justice, randomization is the fairest and most ethical way to determine which patients receive (comprehensive) care that will certainly increase convenience and access to experienced primary caregivers and is expected to improve outcome based on all that is known about medical homes. Though they will not receive comprehensive care, usual care patients will not receive care that is less available or effective than the care that would be provided in the absence of the project;

3. The issue under investigation cannot be well assessed using less rigorous designs. Use of the study design often used in evaluating in different approaches to health care delivery – a simple before-and-after comparison to assess costs and outcomes before and after introducing comprehensive care—would entail major potential biases, including regression to the mean (with children who have the highest hospital costs in one year being likely to have lower costs in the following year, independent of any effect of the program). Randomization helps to ensure that the children in the usual care and comprehensive care groups will have similar baseline risks and that the results of the project will be valid, more likely to be published in widely read journals, more convincing to physicians, health policy analysts, and third-party payers, and thus more likely to produce appropriate changes in pediatric health care delivery.

**Treatment:** Children will be randomized to either usual care (the care that would be provided in the absence of this project) or comprehensive care.

**Usual care:** This is care that is routinely provided by the pediatric residents and faculty of UT Health at present. Under this, children currently receive care in the general pediatric clinic with consultation from
Comprehensive care: This care will be provided in a new clinic (the High-Risk Children Clinic) that is now being established in a dedicated area of the UT Professional Building. Patients will receive care for chronic illnesses, acute illnesses, complications, or exacerbations, and social support from a small team of dedicated care givers in this clinic (currently planned to include 2 pediatricians, 2-3 pediatric nurse practitioners (PNPs), and a social worker). We expect this clinic to provide care for 350 high-risk children. The additional features of comprehensive care are:

1. Mothers will have 24/7 access to one of the PNPs or pediatricians by phone (through a pager) or in the clinic.
2. When the staff are contacted on weekdays, children needing prompt medical attention will be seen within 8-16 hours (depending on the time when the staff are first contacted).
3. When the staff are contacted over the weekend, such children will be routinely seen the following Monday morning.
4. Emergencies at night and on the weekend will be referred to the ED.
5. Our staff will speak with the responsible physicians for patients who need to be seen in the ED or admitted to the hospital.
6. Our staff will also contact the parents the morning following any ED visit and will also formally consult on the patients after admission to the hospital, in part to ensure timely follow-up as needed.
7. To the extent possible, the care practices will be evidence-based and determined based on consultations as needed with pediatric subspecialists and on the best available evidence in the medical literature, particularly Cochrane systematic reviews of clinical trials.
8. Written guidelines and procedures will be prepared for treating common problems. Children will receive comprehensive care for at least one year and until their health care problems improve and they are judged to no longer require such care.
9. Once the clinic reaches capacity, discharge of these patients to receive usual care in the general pediatric care will allow enrollment of new children needing comprehensive care.

Informed consent: We believe that this proposal meets the requirements of waiver of documentation of consent under federal regulations (45 CFR 46.117c), as proposal presents no more than minimal risk of harm to participants and involves no procedures for which written consent is normally required outside of the research context. For the following reasons, we propose to seek verbal consent with permission from CPHS to waive documentation of consent:

1. The primary issue under investigation is not whether the intervention is beneficial but whether it is sufficiently cost effective to be affordable and sustainable;
2. There are no discernible risks, experimental therapies or procedures that require written consent in clinical practice and the project does not violate the rights or welfare of the participants. Indeed, as noted above the intervention increases convenience and access to care from highly committed and experienced caregivers and is expected to reduce risk and improve outcomes based on a large body of evidence from studies of medical homes;
3. We will provide written information about the project to all participants before obtaining their verbal consent. Parents will be periodically reminded that their child is in a project when they receive modest financial incentives ($10 gift certificate) after they are contacted by phone to determine whether their child has been hospitalized in the prior 3 months;
4. In a society in which signatures are ordinarily required only when accepting greater obligation, risk, liability, or cost, the requirement for written consent for this specific project may be misleading.

Data collection: The data needed to assess the above hypotheses will be collected by a research nurse and a health care economist.

1. We will obtain Medicaid data for the health services and the reimbursements provided to participants from Texas Health and Human Services.
2. We will speak with the parent(s) of participants by phone or in person every 3 months to ask about ED visits and hospitalizations.
3. We will review records if needed at other Houston hospitals (e.g., Texas Children’s Hospital, the only other Houston hospital were pediatric intensive care is likely to be provided.). We will obtain authorization from the parent for release of records.

To the extent possible, any discrepancies between parent report, hospital records, and Medicaid record will be resolved and adjudicated by an investigator unaware of the particular patient’s treatment group (Drs. Tyson or Evans). Because of the information to be provided by Health and Human Services, review of medical records is not likely to be required to identify serious illness (prolonged hospital stay, PICU admission, or death) or major costs among Medicaid patients (expected to account for the majority of our patients, particularly those who experience serious illness).

To help ensure that the parent(s) will answer their telephones and speak with us when we call, we will send them a $10 gift certificate for each call.

Economic Analyses: The primary economic evaluation will be conducted from the healthcare system perspective. We will include all direct medical costs for inpatient and outpatient services received by each patient during follow-up care. Inpatient costs will be estimated by multiplying MHH charges by department-specific cost-to-charge ratios obtained from the hospital's annual Medicare Cost Report. Outpatient costs will be estimated using an RVU-based methodology. The appropriate resource-based RVUs for each utilized clinic service will be obtained from the UT-P billing system and will then be multiplied by the applicable Texas Medicaid conversion factor. In addition, the salary supplements given primary caregivers for taking telephone calls after clinic hours will be added to the costs of comprehensive care. We will also estimate costs for resources utilized outside this site by obtaining from parents the number and types of services used and if necessary, will seek the outside medical records. We will then impute costs for inpatient care that occur outside MHH by multiplying the sample’s observed median MHH cost per both non-critical bed-day and PICU bed-day by the reported outside length of stay in a non-critical care unit and intensive care unit, respectively. The costs for outpatient care received outside this site will be obtained by multiplying the reported number of clinic services by the estimated cost for these services at this site.

A secondary economic evaluation, from a third-party payer (government) perspective, will be conducted by using Medicaid data on all reimbursements and resources utilized by the participants covered by Medicaid only during follow-up. Such data will be obtained from the Texas Health and Human Services.

An additional analysis, from the hospital perspective, will also be conducted to assess the financial impact of the intervention on MHH by assessing the difference between revenues and costs (i.e. net gain or loss) associated with providing acute care for this population. We will also assess the
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opportunity costs of using hospital beds for caring for high-risk children that could instead be used for children in the general population, by subtracting the median hospital’s net income (per bed-day) associated with pediatric hospitalizations in general from that of providing acute care for participants in this project.

Lastly, the intervention’s financial impact on the medical school will also be evaluated by assessing the difference between revenues and expenses associated with running the high-risk clinic and the amount of staff time that will be freed up from reduced hospitalizations. Such assessment will require a time motion study of faculty time devoted to high-risk children on rounds in the pediatric floor, PICU, and in the high-risk clinic.

For all the economic analyses, regardless of the perspective adopted, costs will be inflated to 2013 U.S. dollars using the Consumer Price Index for medical care and will be discounted at 3% per year. Finally, the incremental cost per episode of serious illness averted by comprehensive care vs. usual care will be computed. In case comprehensive care reduces costs without increasing or decreasing serious illness, the actual net savings associated with the intervention will be calculated and reported. We will then examine the robustness of the results by performing univariate sensitivity analyses of plausible ranges for the project parameters and also probabilistic sensitivity analysis using Monte Carlo simulation.

Statistical Analyses: Standard frequentist analyses will be performed at the conclusion of the project. An intent-to-treat approach will be used in all analyses. We will fit generalized linear mixed models to account for within patient correlation (due to multiple ED visits, ICU days etc.) as well as variable follow-up time. As recently recommended,3 Bayesian analyses will also be performed.4 (These analyses will be performed by Claudia Pedroza, PhD, a Bayesian statistician in the Center for Clinical Research and Evidence-Based Medicine, who recently explained their rationale in a trial she coauthored in the New England Journal of Medicine.5) In contrast to frequentist analyses, Bayesian analyses can provide a direct estimate of the probability that comprehensive care reduces serious illness in high-risk children by specified amounts. A 95% credibility interval (analogous to frequentist confidence intervals) will be included for each estimate. The probability of benefit can be estimated at intervals (without adjusting final credibility intervals for repeated analyses) during the project. The need to provide outcome and cost data to Texas Health and Human Services by the end of the 2nd year of funding can be met by such analyses. Interim Bayesian analyses can also be used in seeking additional funding to be used.

Sample Size and Power. This project will be conducted to meet the highest standards,6,7,8 including the standards for sample size determination. A predetermined sample size cannot be selected at this point because of uncertainty about proportion of children who will experience serious illness in the usual care group. For this reason, the sample size will be determined after we have 12 month data for a cohort of children (those enrolled in the first year). The sample size will be determined by a statistician based solely on the data for the usual care group without knowledge of the data for the intervention group. Alpha error = 0.20; beta error = 0.80; reduction in serious illness = 33%). The investigators are committed to completing the project with the full sample size.
REFERENCES


4 Speigelhalter DJ, Abrams KR, Myles JP. Bayesian Approaches to Clinical Trials and Health Care Evaluation. Chicester: Wiley; 2004


